NASDAQ: BTAI

Virtual R&D Day:
Revolutionizing Drug Discovery and Development Through AI

September 23, 2021
12-2 pm ET

Forward-Looking Statements

This presentation and the accompanying oral statements include "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements in this presentation and the accompanying oral statements include but are not limited to the advancement and development of BXCL501 and BXCL502, the potential benefits of BXCL501 and BXCL502, potential commercialization and related strategy for BXCL501, our use and the potential of AI, the potential of BXCL501 and BXCL502 in combination with each other, the clinical development timeline of our product candidates, our potential medical setting and geographic expansion, and other information that is not historical information. When used herein, words including "anticipate," "being," "will," "plan," "may," "continue," "intend," "designed," "upon," and similar expressions are intended to identify forward-looking statements. In addition, any statements or information that refer to expectations, beliefs, plans, projections, objectives, performance, or other characterizations of future events or circumstances, including any underlying assumptions, are forward-looking. All forward-looking statements are based upon BioXcel's current expectations and various assumptions. BioXcel believes there is a reasonable basis for its expectations and beliefs, but they are inherently uncertain.

BioXcel may not realize its expectations, and its beliefs may not prove correct. Actual results could differ materially from those described or implied by such forward-looking statements as a result of various important factors, including, without limitation, its limited operating history; its incurrence of significant losses; its need for substantial additional funding and ability to raise capital when needed; its limited experience in drug discovery and drug development; its dependence on the success and commercialization of BXCL501 and BXCL701 and other product candidates; the failure of interim and preliminary data from its clinical studies to predict final study results; failure of its early clinical studies or preclinical studies to predict future clinical studies; its ability to receive regulatory approval for its product candidates; its ability to enroll patients in its clinical trials; undesirable side effects caused by BioXcel's product candidates; its approach to the discovery and development of product candidates based on EvolverAI is novel and unproven; its exposure to patent infringement lawsuits; its ability to comply with the extensive regulations applicable to it; impacts from the COVID-19 pandemic; its ability to commercialize its product candidates; and the other important factors discussed under the caption "Risk Factors" in its Quarterly Report on Form 10-Q for the quarter ended June 30, 2021, as such factors may be updated from time to time in its other filings with the SEC, which are accessible on the SEC's website at www.sec.gov and the Investors section of BioXcel's website at www.bioxceltherapeutics.com.

These and other important factors could cause actual results to differ materially from those indicated by the forward-looking statements made in this presentation. Any such forward-looking statements represent management's estimates as of the date of this presentation. While BioXcel may elect to update such forward-looking statements at some point in the future, except as required by law, it disclaims any obligation to do so, even if subsequent events cause our views to change. These forward-looking statements should not be relied upon as representing BioXcel's views as of any date subsequent to the date of this presentation.

Certain information contained in this presentation relates to or is based on studies, publications, surveys, and other data obtained from third-party sources and BioXcel's own internal estimates and research. While BioXcel believes these third-party sources to be reliable as of the date of this presentation, BioXcel has not independently verified, and BioXcel makes no representation as to the adequacy, fairness, accuracy, or completeness of any information obtained from third-party sources. In addition, market data included in this presentation involves a number of assumptions and limitations, and there can be no guarantee as to the accuracy or reliability of such assumptions. While BioXcel believes its own internal research is reliable, such research has not been verified by any independent source.

Agenda and Speakers

- Our Corporate Strategy
- R&D at BioXcel Therapeutics
- Applying AI Ecosystem to BTI Drug Discovery and Development
- New Product Concept Strategy
- Major Depression: Focus on Anxiety
- Expansion of BXCL501 Development Into Depression
- Closing Remarks
- Q&A

**Dr. Berman is a paid consultant to BioXcel*

Vimal Mehta, Ph.D.
Founder and CEO

Frank Yocca, Ph.D.
Chief Scientific Officer

Friso Postma, Ph.D.
Senior Director,
Neuroscience and AI

Michael De Vivo, Ph.D.
Vice President,
Neuroscience

Robert Berman, M.D.*
Adjunct Professor of Psychiatry,
Yale University School of Medicine

Robert Risinger, M.D.
Senior Vice President,
Clinical Development

Our Corporate Strategy

Vimal Mehta, Ph.D.
Founder and CEO

Video: *Revolutionizing Drug Discovery and Development Through AI*

Our Mission

Develop transformative medicines utilizing AI approaches in Neuroscience and Immuno-oncology

Neuroscience

Symptoms from stress-related behaviors

BXCL501

- **Schizophrenia**-related agitation
- **Bipolar Disorder**-related agitation
- **Dementia**-related agitation
- **Delirium**-related agitation
- **Opioid withdrawal** symptoms

Immuno-Oncology

Innate Immunity

BXCL701

- **Aggressive form of prostate cancer**
- **Advanced solid tumors**

Proprietary & Confidential

Corporate Neuroscience Strategy

From First-in-Human Trial to acceptance of our NDA for BXCL501 in Under 3 Years

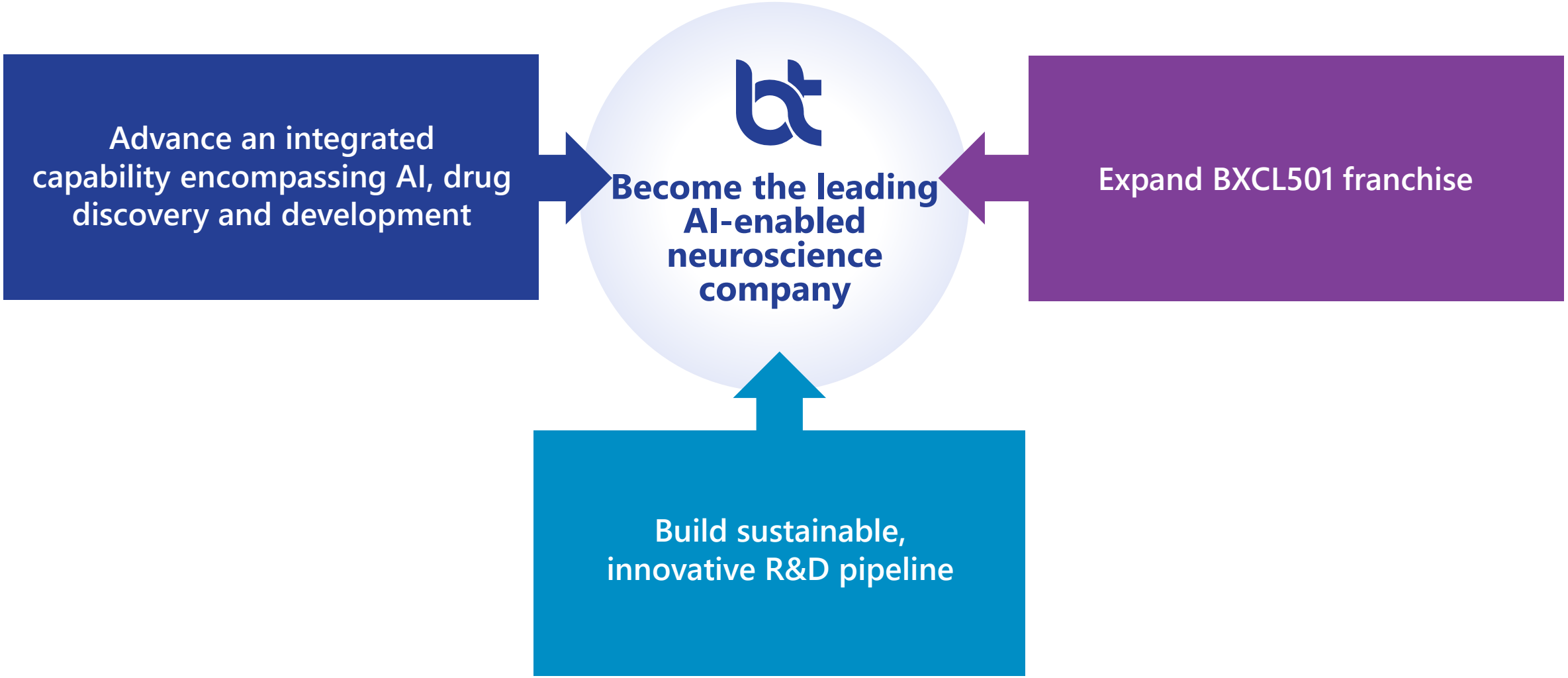
Utilizing a robust AI platform to develop transformative medicines

Focusing on hard-to-treat neuropsychiatric symptoms by delineating underlying human biology and behavioral pathways

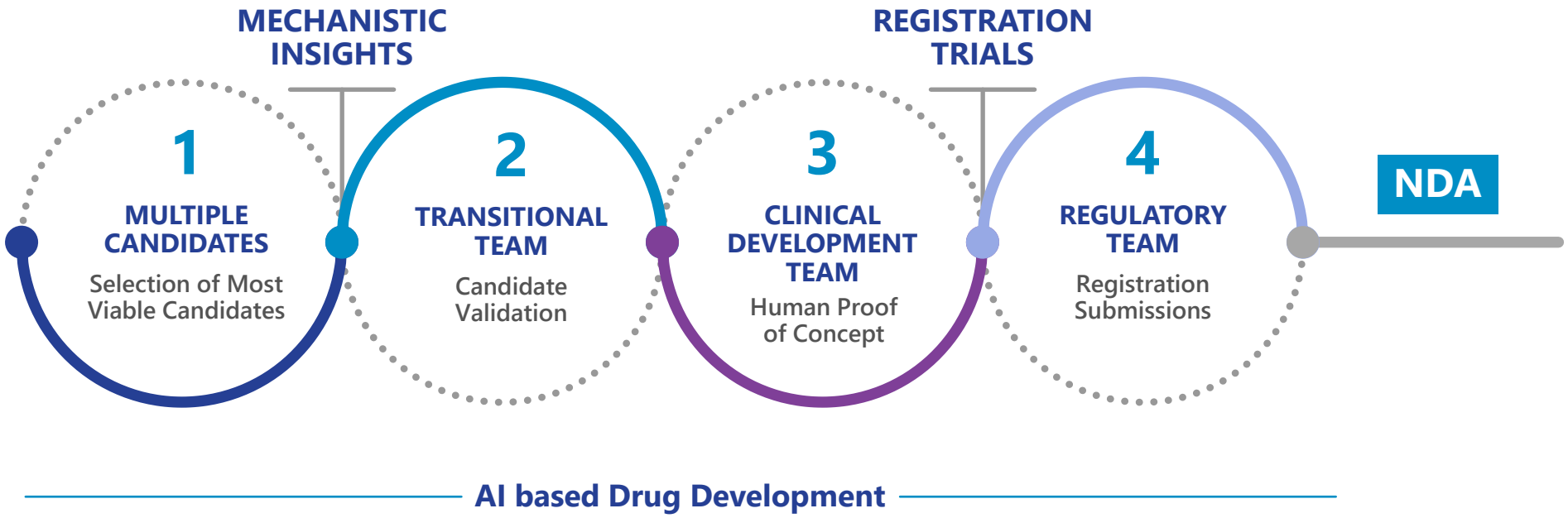
Leveraging re-innovation of clinical (NCEs) candidates and approved drugs

Delivering long-term stakeholder value through sustainable R&D pipeline

Five-Year Vision for Growth



Strategy for Accelerating Development Cycle through AI



Potential to:

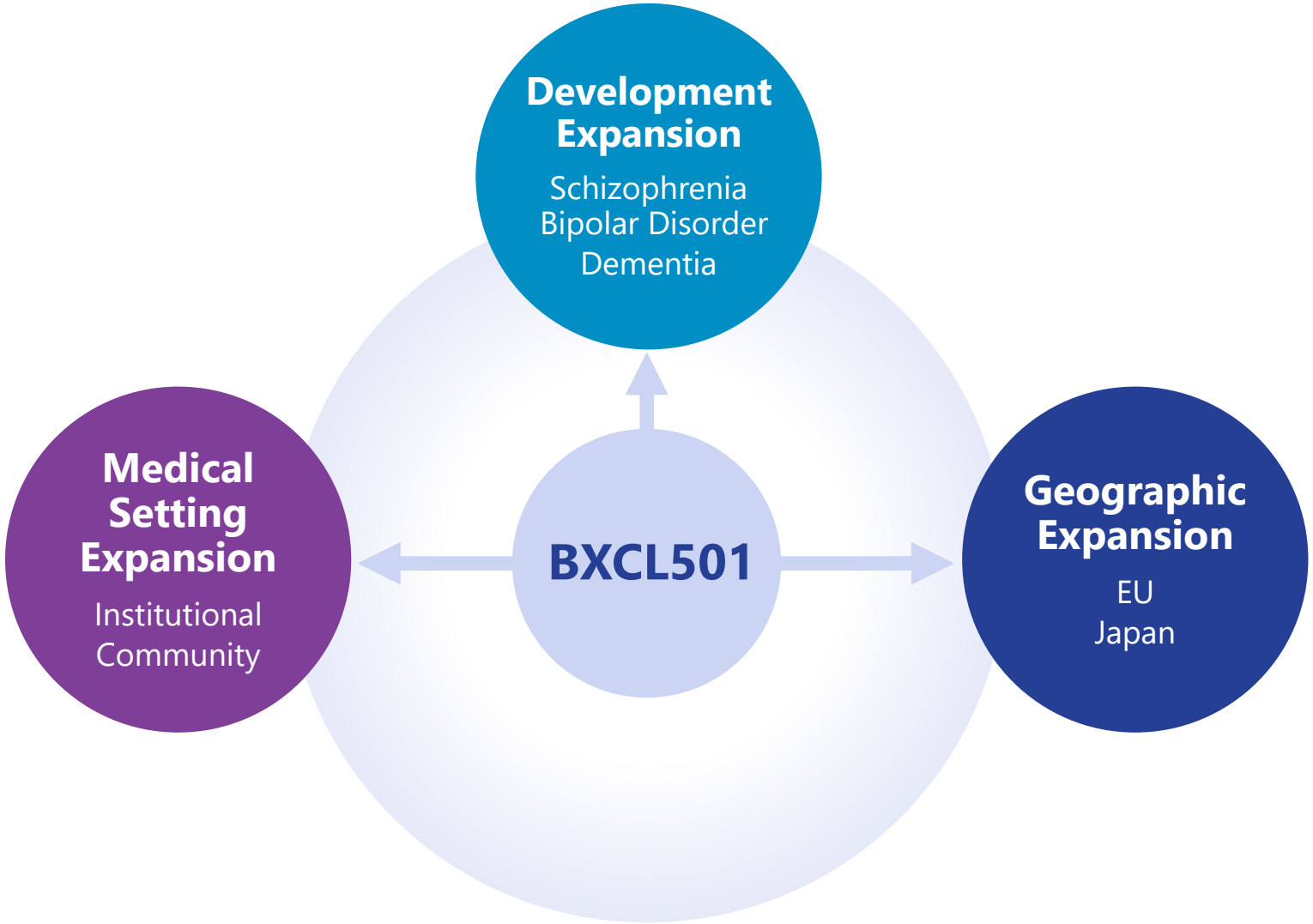
- Optimize R&D Economics
- Shorten Development Timelines
- Achieve Greater Probability of Success

Parent AI Platform Validated by Multiple Global Partners

Over 1.5 decades of experience working with 100+ biopharma companies

- **Multiple Pipeline Candidates (>10) Generated**
  - NDA
  - Late Stage Clinical
  - Early Stage Clinical
  - Preclinical Stage
- **Modalities**
  - Small Molecules
  - Biologics
  - siRNA
  - Gene Editing
  - mRNA
  - Peptides
- **Multiple Therapeutic Area Experience**

Significant Portfolio Expansion Opportunity for BXCL501



Key Clinical and Commercial Catalysts for 2021

Strong cash position $273.1M to fund key milestones*

NEUROSCIENCE – BXCL501

Schizophrenia & Bipolar:

- ✓ NDA submitted in March 2021
- ✓ NDA accepted for filing – PDUFA date of 1/5/2022
- • MAA submission to EMA anticipated in 2H 2021

Dementia:

- ✓ Reported positive topline results from the TRANQUILITY Phase 1b/2 trial
- ✓ TRANQUILITY 40 mcg supplemental cohort study underway
- • EOP2 meeting with the FDA held in 2Q 2021; additional meetings planned in 2H 2021 to discuss Phase 3 program
- • Expected initiation of Phase 3 studies in Q4 2021

Opioid Withdrawal Symptoms:

- ✓ Reported topline results for RELEASE Phase 1b/2 trial

COMMERCIAL STRATEGY

Commercial Readiness Preparations

- ✓ Payer strategy development underway
- ✓ Launched disease education campaign
- • Sales Force size, structure, and operations infrastructure being finalized
- • Sales Force recruitment

IMMUNO-ONCOLOGY – BXCL701

Aggressive Form of Prostate Cancer (cold tumor):

- ✓ Met prespecified efficacy bar in ongoing adenocarcinoma Phase 1b/2 trial
- • More complete efficacy data expected to be presented at a medical conference later this year

Solid Hot Tumors – Basket Trial:

- • Efficacy data readout expected 2H 2021

bioxcel therapeutics®

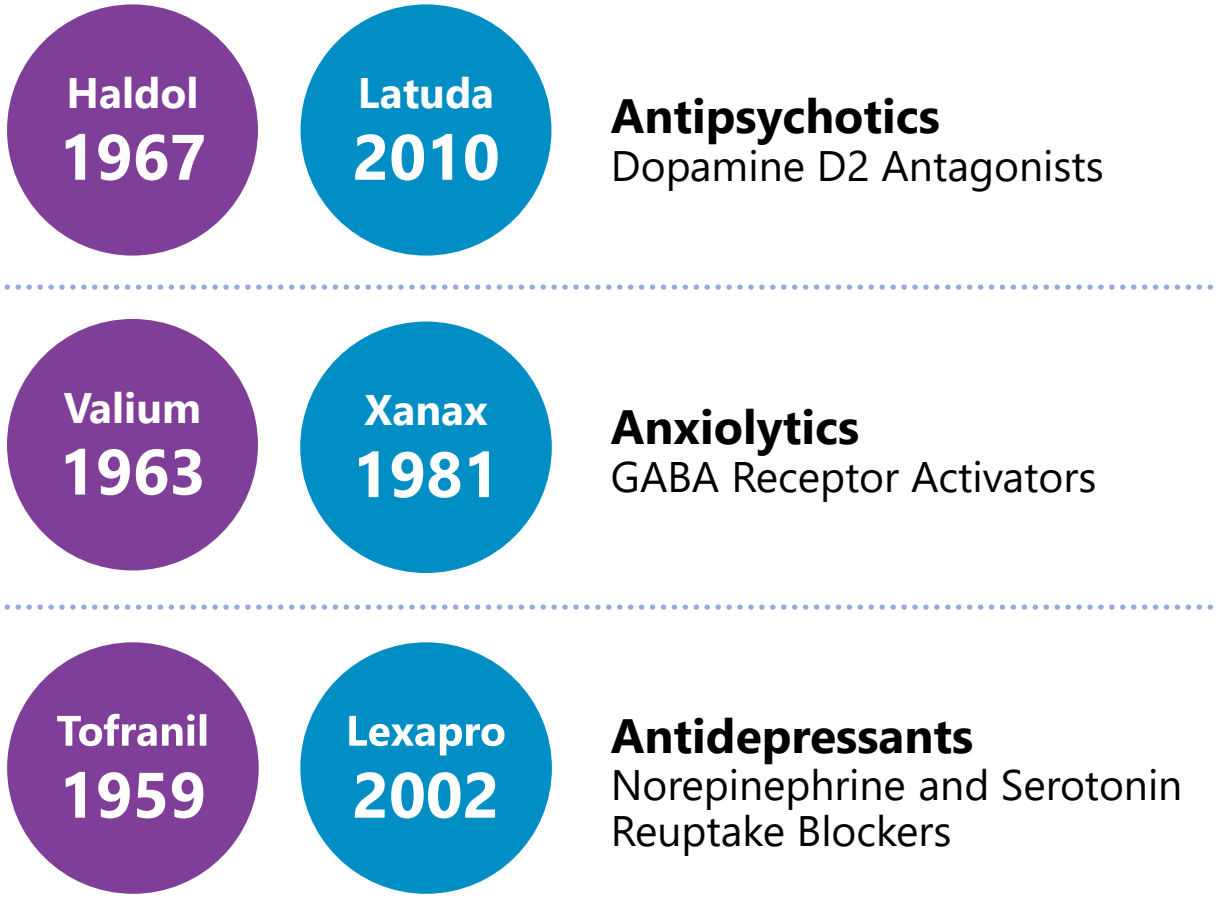
*Reported cash position as of June 30, 2021

R&D at BioXcel Therapeutics

Frank Yocca, Ph.D.
Chief Scientific Officer

CNS Drug Development Requires New, Innovative Approaches

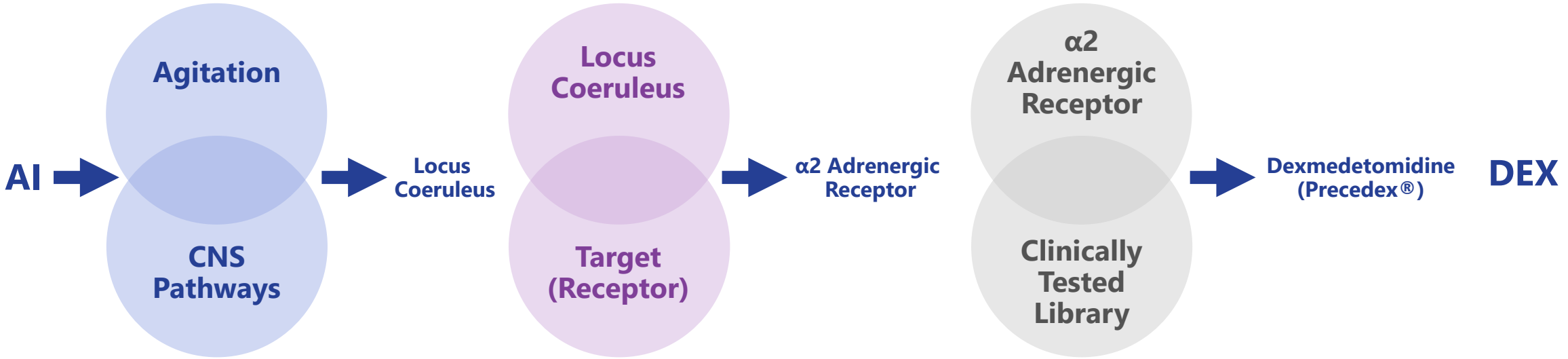
- Currently prescribed psychiatric drugs offer little advantage over original agents discovered 60 years ago
- Reasons for lack of innovation:
  - Poor understanding of CNS pathways
  - Knowledge gap in how brain receptors, circuits modulate symptoms
  - **Linking receptor function to circuits, neuronal pathways and symptoms requires innovative approach (AI)**



The Importance of Treating Specific Symptoms in Mental Illnesses

- Psychiatric illnesses are syndromes made up of a collection of symptoms
- Current medications don't cure psychiatric illnesses or collection of symptoms
- Hard-to-treat symptoms occur in various psychiatric illnesses
- Our AI approach focuses on integrating drug targets, pathways, circuitry, and compounds into new concepts for treating symptoms
- ✓ An example of this approach is BXCL501

BXCL501 AI-Enabled Development Journey

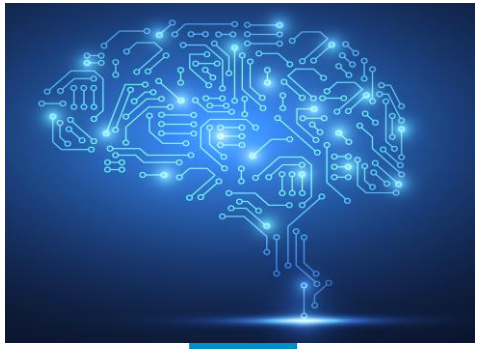


Focus on discovery of novel pathways for stress-related symptoms

Build sustainable, innovative pipeline of CNS drug candidates

Unique AI Approach: New Product Concepts with Focus on the Stress Axis

STRESS



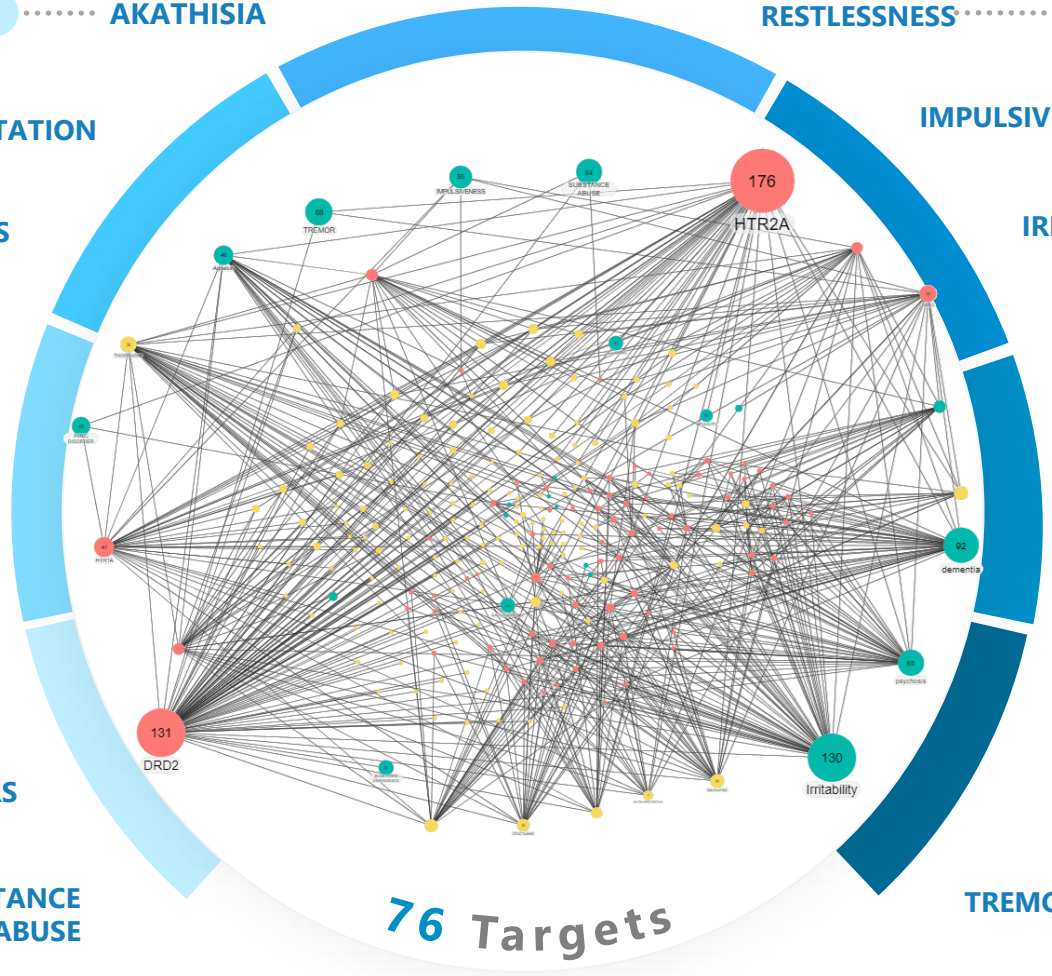
Stress alters the function and activity of CNS pathways

Changes in CNS pathway function and activity of CNS pathways

MACHINE LEARNING CONNECTS:

- **Behaviors (symptoms)**
- **Stress pathways**
- **Drug targets**
- **Drugs**

Universe of Stress-related Symptoms, Targets & Drugs: Dynamic Connectivity Map



2 Targets | 32 Drugs
HALLUCINATIONS

1 Target | 9 Drugs — **AKATHISIA**

RESTLESSNESS — 22 Targets | 5 Drugs

44 Targets | 5 Drugs — **AGITATION**

IMPULSIVENESS — 6 Targets | 43 Drugs

1 Target | 9 Drugs — **PSYCHOSIS**

IRRITABILITY — 59 Targets | 10 Drugs

1 Target | 31 Drugs — **DELUSIONS**

MOOD CHANGES — 3 Targets | 1 Drug

46 Target | 41 Drugs — **DEMENTIA**

PANIC DISORDER — 4 Targets | 29 Drugs

1 Target | 1 Drug — **EMOTIONAL DISTRESS**

SOCIAL ANXIETY — 1 Target | 4 Drugs

3 Targets | 1 Drug — **SLEEP DISORDERS**

SUICIDAL BEHAVIOUR — 1 Target | 15 Drugs

3 Targets | 78 Drugs — **SUBSTANCE ABUSE**

TREMOR — 3 Targets | 40 Drugs

BTI R&D Day Speakers

Friso Postma, Ph.D.
Senior Director, Neuroscience and AI

Applying AI Ecosystem to BTI Drug Discovery and Development

Mike De Vivo, Ph.D.
Vice President, Neuroscience

New Concept Strategy

Rob Risinger, MD
Senior Vice President, Neuroscience Clinical

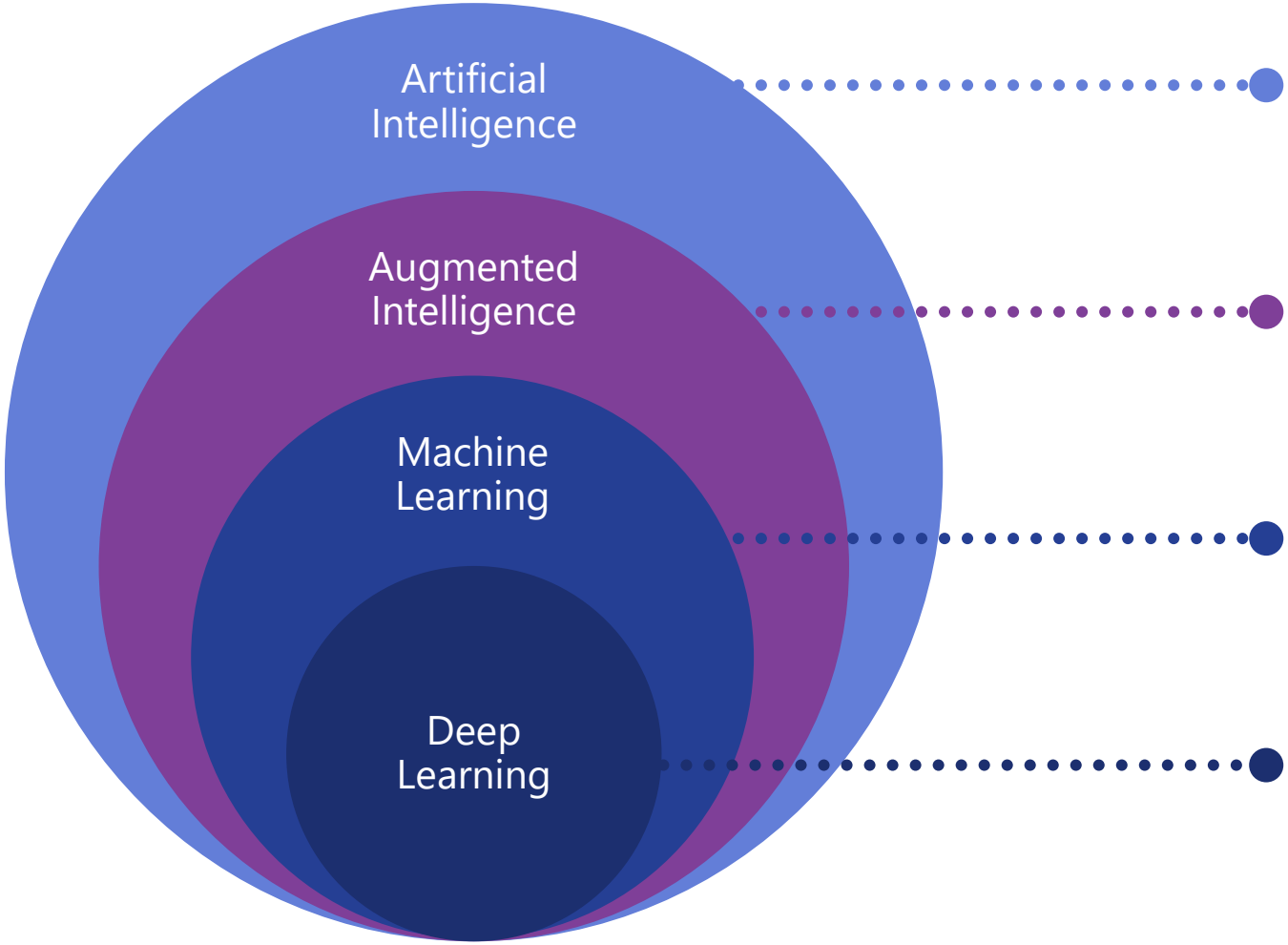
Expansion of BXCL501 to Depression

Applying our AI Ecosystem to Drug Discovery and Development

Friso Postma, Ph.D.

Senior Director, Neuroscience and AI

What is Artificial Intelligence?



A field of science aiming to create algorithms and machines that can mimic intelligent behavior

Using algorithms and machines to augment human behavior and decision making

Algorithms that can learn from data in a supervised or unsupervised manner and improve with experience

Subset of machine learning that uses algorithms organized as multi-layer neural networks

Augmented Intelligence Addresses Critical Barriers in Neuroscience

GENERATE INSIGHTS FROM BIG DATA

PubMed
DRUGBANK
PubChem
hetio/hetionet
Hetionet: an integrative network of disease
UniProt
ChEMBL

ADDRESS COMPLEX CIRCUITRY

Link Prediction

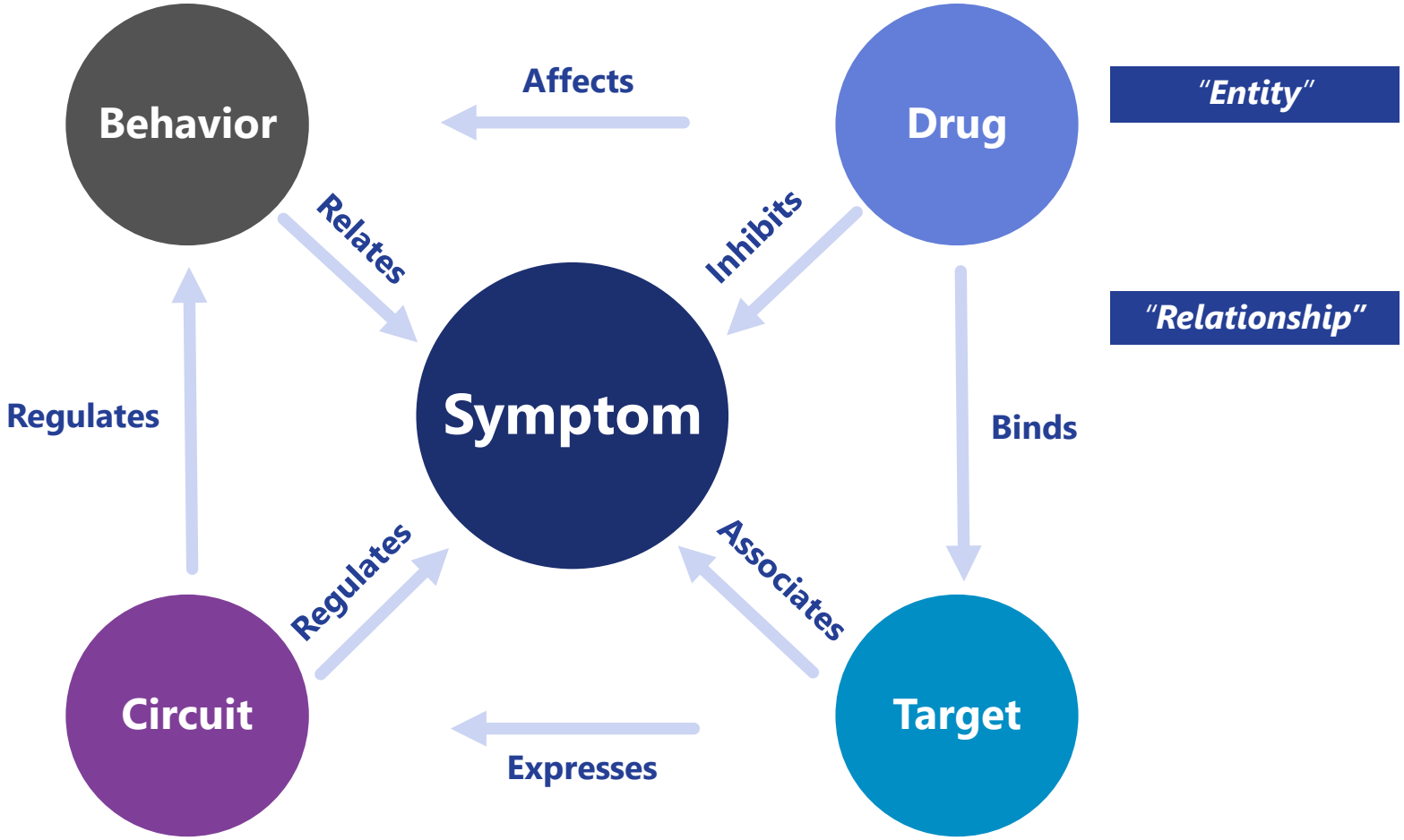
Behavior
Target
Drugs

QUANTIFY BEHAVIOR

Integrated Multidisciplinary Global Team

BioXcel Discovery Centers on the Knowledge Graph

Connecting Relevant Drug Targets to CNS Pathways in a Human Biology-Centric Manner



Product Concept Generation: Linking Circuit to Behavior

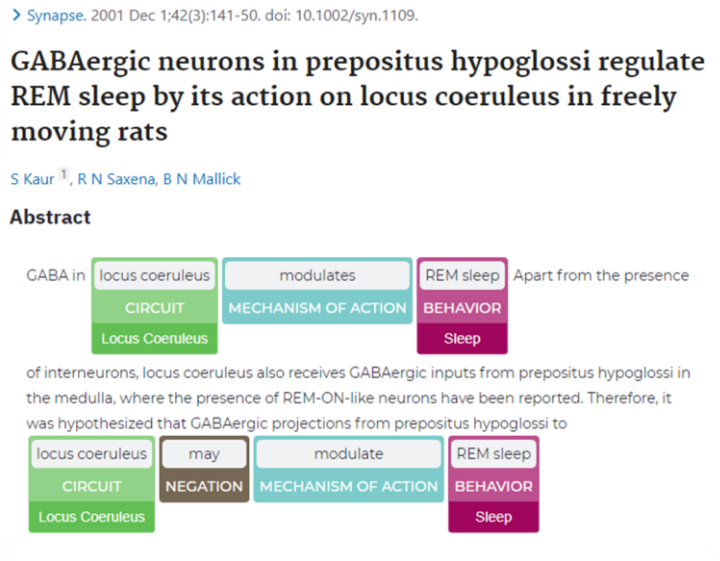
Find entities of interest in text and how they are related. For example: **"Receptor" – "Activates" – "Neuron"** (AI)

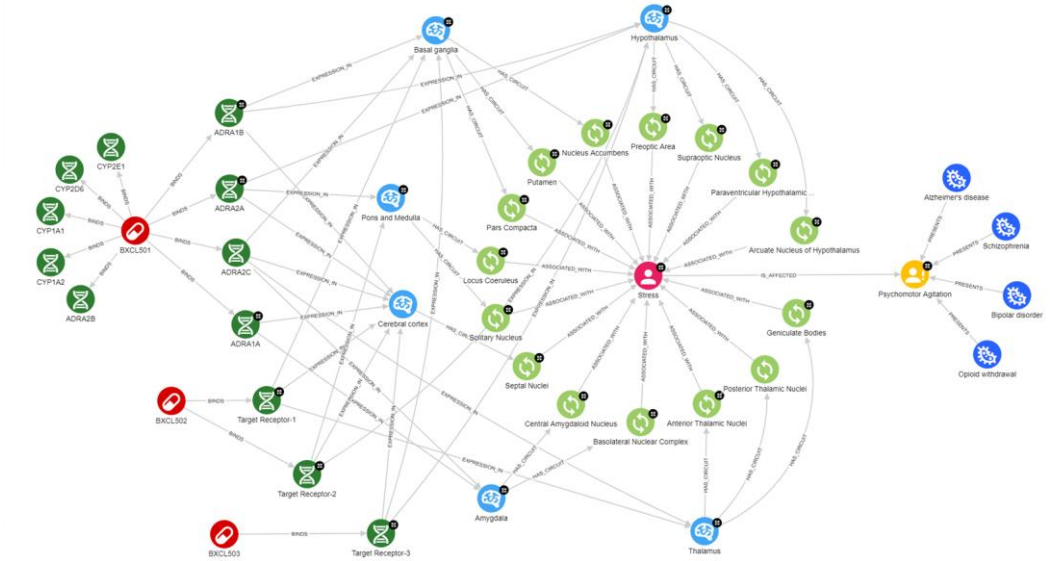
The KG links **Compounds** - **Targets** – **Circuits** – **Behavior**

Insights Engine is used to interrogate the Knowledge Graph (AI)

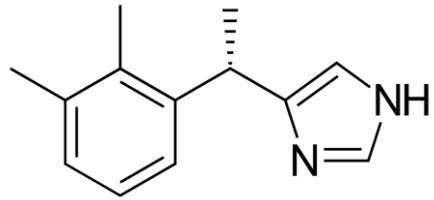
Concepts are evaluated in an opportunity prioritization model

NLP pipeline → Knowledge Graph (KG) → KG Data Science / KG exploration → Concept Generation





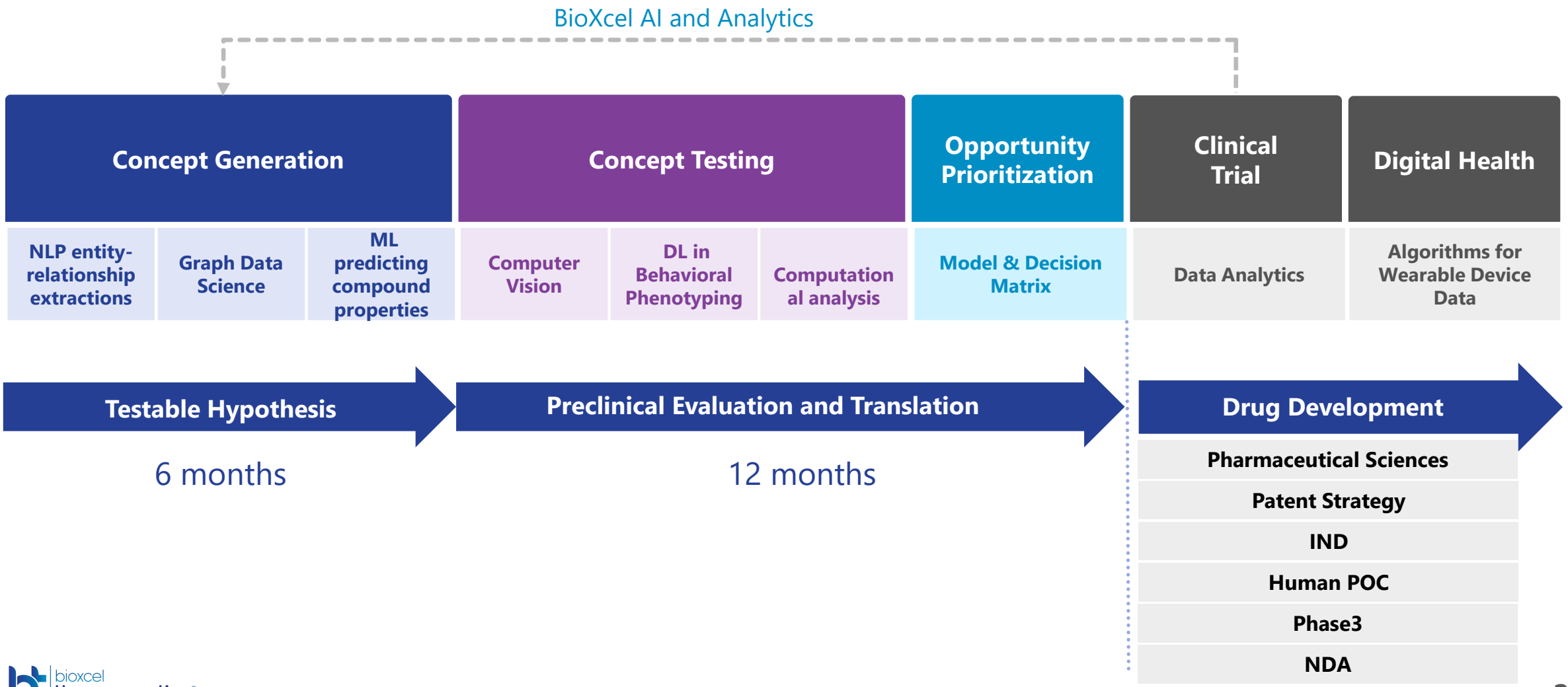
"Dexmedetomidine for Agitation"



Video: *Knowledge Graph Discovery Path Linking Agitation to BXCL501*

AI Ecosystem: Strategy for Accelerating the Drug Re-Innovation Process

From Product Concept to First-in-Human

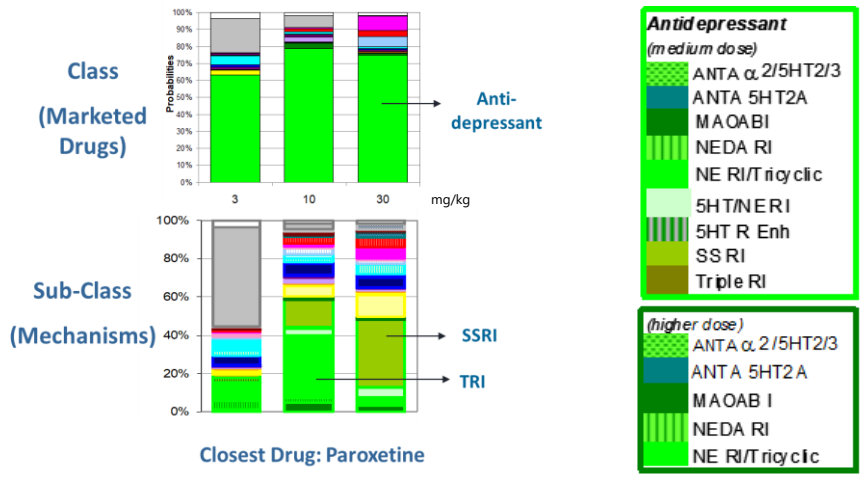


AI-based Preclinical Concept Evaluation

Quantitative Mouse Behavioral Phenotyping using SmartCube®*

- Multidimensional profiles of both *passive* and *reactive* mouse behavior
- >1000 features extracted using **AI (computer vision)**.
- Reference data-bases link behavioral features of existing drugs to compound activity

Example Compound



*Psychogenics Inc.

Digital Technologies for Developing Potential Preventative Therapies

Leveraging External Expertise

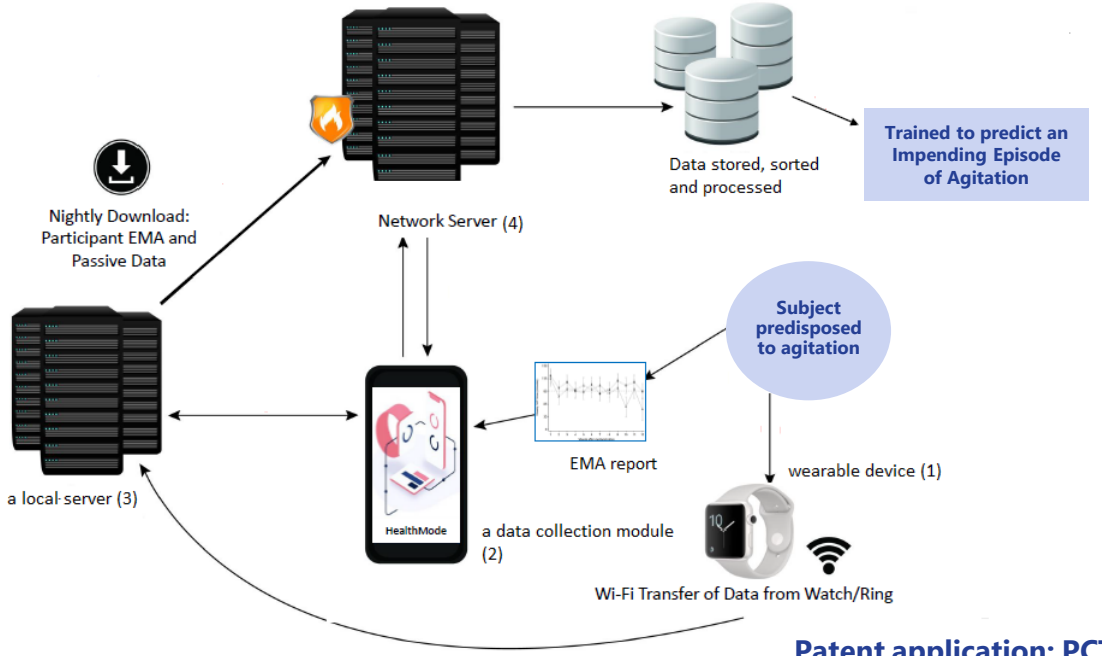
Partnerships:

- **Yale University**

  Investigating biomarkers in agitated patients with schizophrenia

- **BTI/MindMed**
  - Patent filed for infrastructure
  - New study to train algorithms detecting emergence of agitation, expected to begin Q4 2021

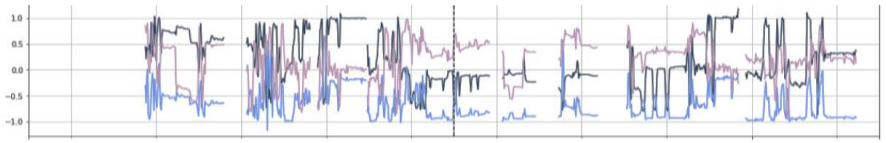
Digital Infrastructure to Remotely Capture Data Associated With Agitation



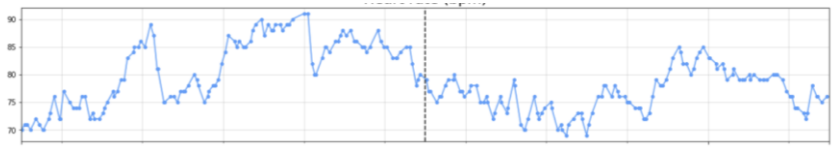
Patent application: PCT/US2020/051256

Data From Patients Successfully Captured

Watch Accelerometry (x,y,z)



Heart Rate (bpm)



New Product Concept Strategy: BXCL502 and Emerging Pipeline

Michael De Vivo, Ph.D.
Vice President, Neuroscience

Focus on Clinically Important Brain Circuits to Generate Innovative Product Concepts

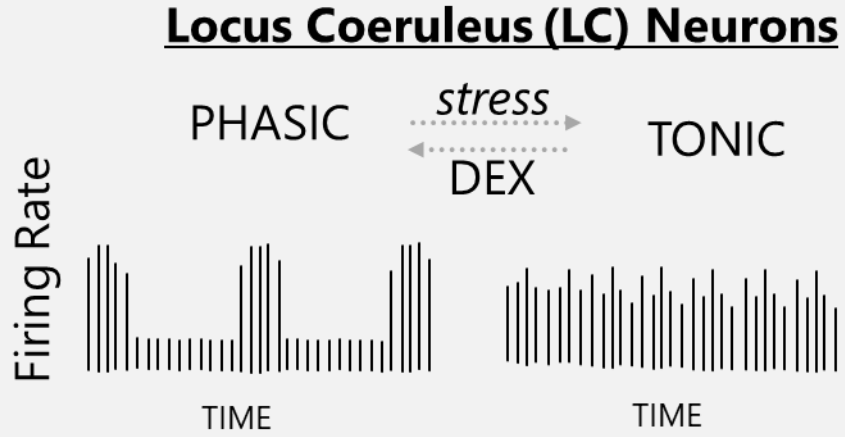
- Over 100 clinically relevant pathways identified using novel technologies
- AI is required to make sense of these new data
- Novel targets on these pathways are matched to drugs in the compound library (1000+)*

*Clinical compounds with established safety profiles likely to penetrate the BBB

Locus Coeruleus — **Norepinephrine** → Amygdala

BXCL501 is designed to target this LC pathway that mediates stress-related behaviors

Addressing Underlying Biology in Neuropsychiatric Symptoms



- LC neurons fire in a phasic mode. When stressed, LC neurons fire in a tonic mode.
- Tonic firing causes anxiety-related behaviors
- Dexmedetomidine potentially reverses this firing pattern and restores phasic activity

- For most neuropsychiatric drugs, the exact pathways that are affected are not known
- BXCL501 targets core causal mechanism of agitation, to help restore normal function to LC neurons and reduce agitation

Finding drugs with the potential to restore normal function to specific pathways is the basis of our innovative and sustainable pipeline

BXCL502 for Chronic Treatment of Agitation

Proposed Indication
Indicated for chronic treatment of agitation in patients with dementia (monotherapy)

Potential Mechanism of Action
Potent and selective antagonist for a GPCR target that affects serotonergic signaling in the cerebral cortex.

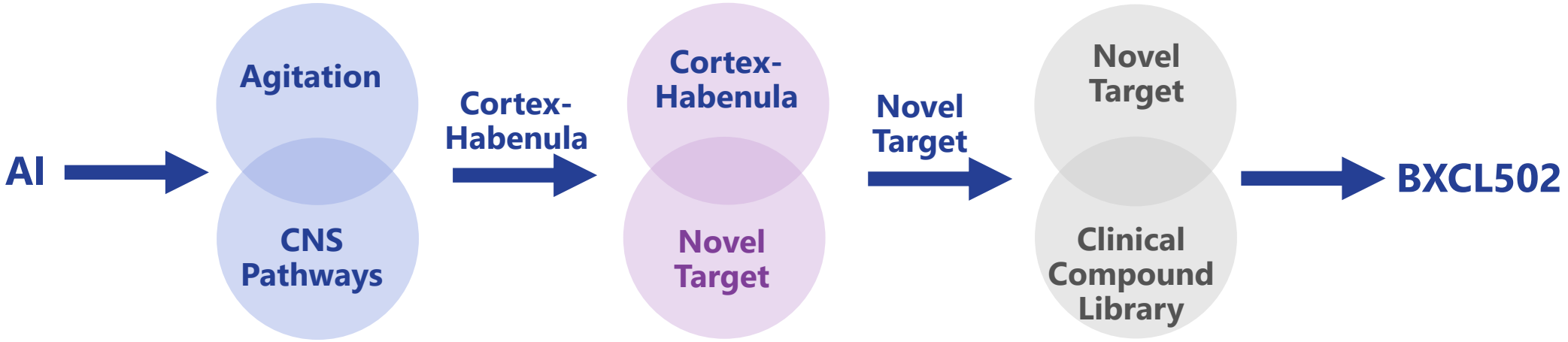
Confidence in Rationale
Demonstrated activity in two animal models

Patient Exposure
Hundreds of patients exposed to the compound for 52 weeks

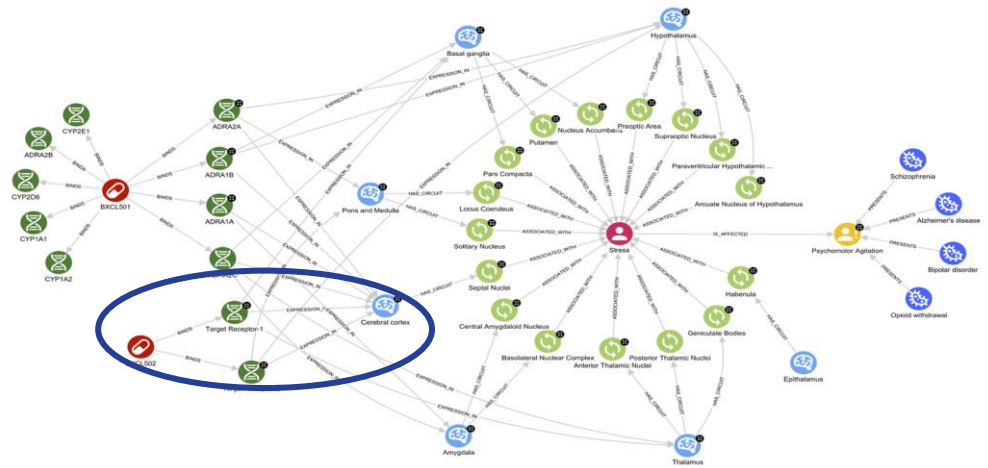
Previous Efficacy Findings
Previously demonstrated improvement in a clinically validated scale used for agitation in 3 clinical studies (secondary endpoint)

Status
BXCL502 Formulation and clinical development planning underway

BXCL502, an AI-Enabled Product Concept

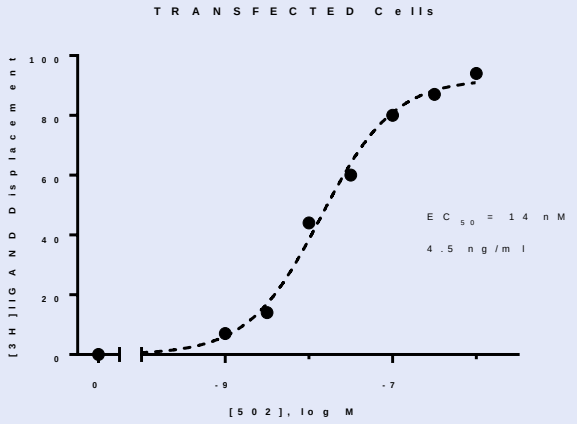


KNOWLEDGE GRAPH: BXCL502



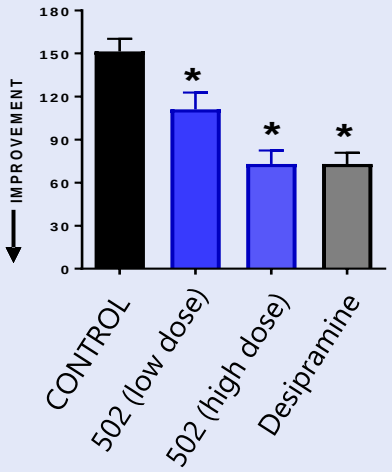
Preclinical Evidence to Support BXCL502 Rationale

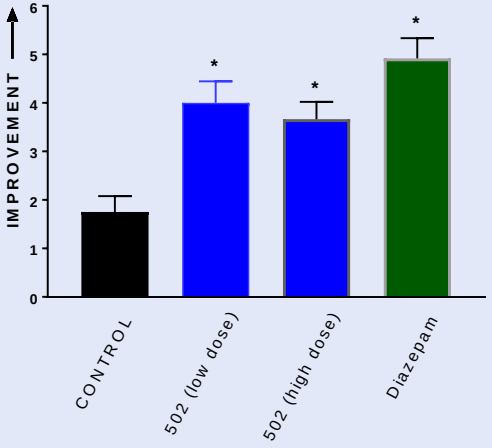
Identified Potential MOA



- Novel potential mechanism for modulating a monoaminergic signaling pathway
- Not an MOA of current anxiolytics, antidepressants, antipsychotics

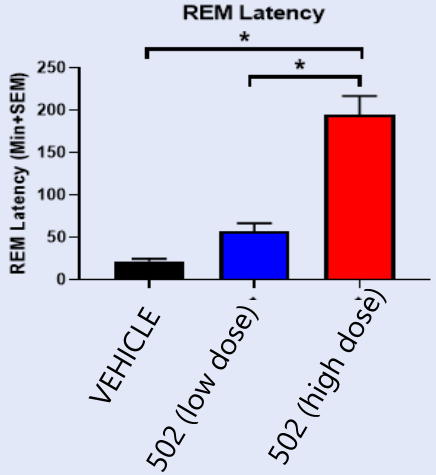
Effective in 2 Stress-Related Animal Models





- Preclinical studies showed robust and potent activity with respect to stress-related behaviors that was comparable to benzodiazepines and antidepressants

Discovered a Translational Biomarker



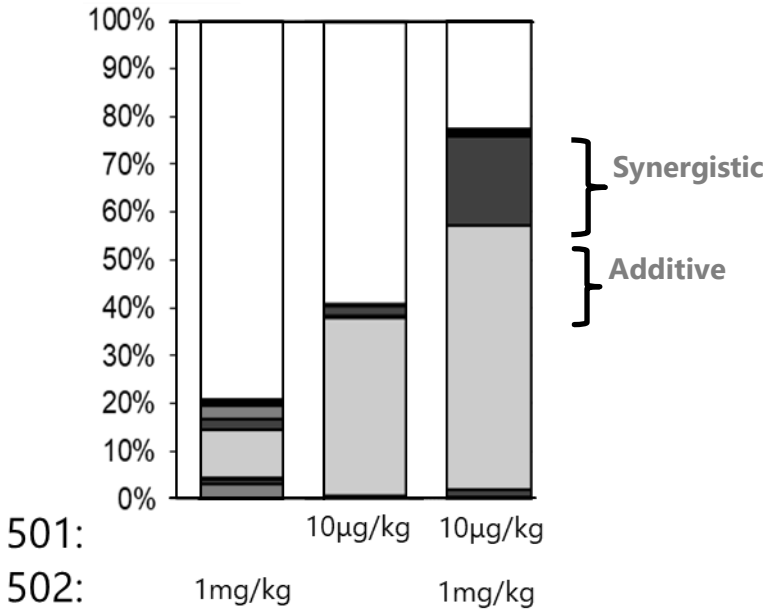
- REM latency is a potential translational biomarker that will be used in effort to get early dosing information from healthy volunteers

Clinical Evidence to Support BXCL502 Rationale

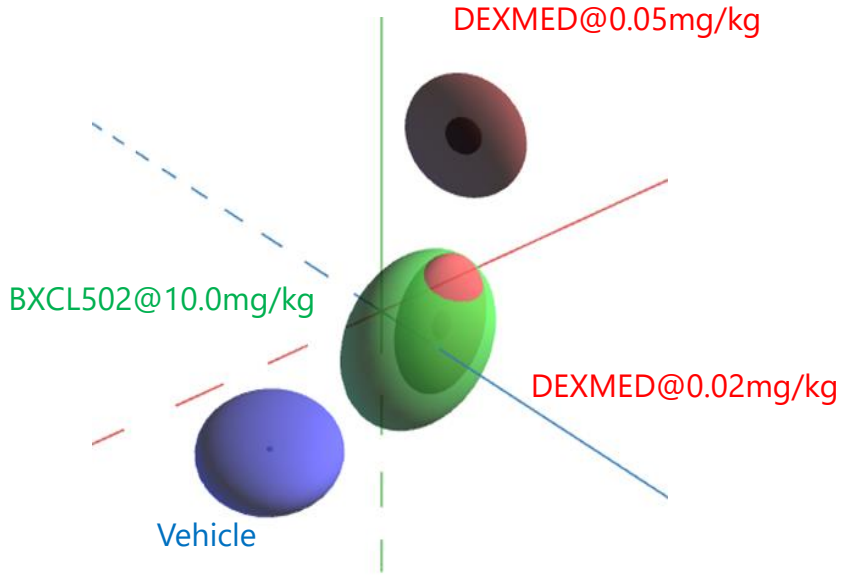
- AI search for clinical data with BXCL502 disclosed prior use in clinical trials in dementia
- BXCL502 was well tolerated and showed early signs of activity in prior clinical trials in the target patient population
- **Clinical data enabled the discovery of this drug through interrogation of the knowledge graph**

Phenotypic Analysis of BXCL502 Suggests Potential Synergy with BXCL501

Low Doses of 501 and 502 Demonstrated Synergism in Phenotypic Assay



Principal Component Analysis Showed Convergence of 501 and 502 on Behaviors

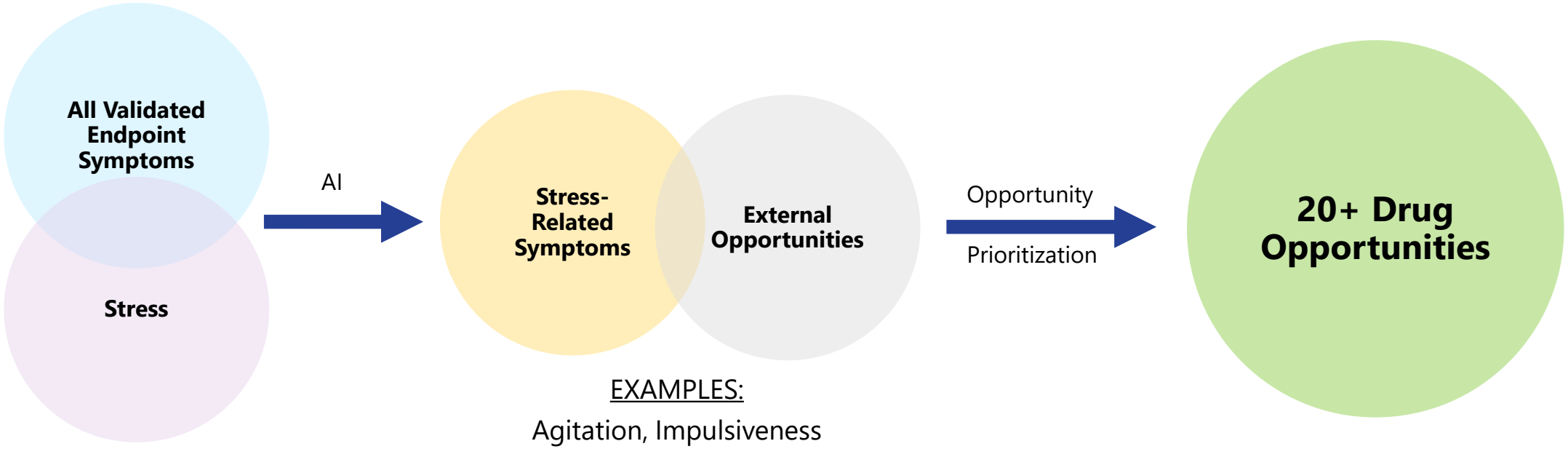


BXCL502, a Differentiated and De-risked Candidate

Pathway	✓ High expression in brain on pathways associated with stress response
Confidence and Rationale	✓ Robust confidence in rationale, based on preclinical studies that showed comparable activity to benzodiazepines and antidepressants
Efficacy Results	✓ Showed improvement in clinically validated endpoint for neuropsychiatric symptoms related to agitation
Human Safety	✓ Generally well-tolerated in hundreds of patients after 52 weeks of dosing
Patent Strategy	✓ Novel formulation strategy under development ✓ Opportunities to expand IP position in combination with complementary mechanisms

- **PROPOSED INDICATION**: Chronic treatment of agitation as a monotherapy in patients with dementia
- Potential for combination therapy with BXCL501

AI-Informed Emerging Pipeline



EXAMPLES:
Agitation, Impulsiveness
Irritability, Delusions
Hallucinations

- Focus on symptoms affected by stress
- Prioritize symptoms that contribute to validated clinical scales like HAMD, PANSS, NPI
- Use an AI-enabled strategy to identify relevant targets and drug opportunities

Major Depression: Focus on Anxiety

Robert Berman, M.D.*

Adjunct Professor of Psychiatry, Yale University School of Medicine

**Dr. Berman is a paid consultant to BioXcel Therapeutics*

Background

Shareholder	✓ Biohaven Pharmaceuticals (co-founder), Osmol Therapeutics, MindImmune Therapeutics, Gilgamesh Pharmaceuticals
Paid consultant	✓ Biohaven Pharmaceuticals, BioXcel Therapeutics, Canaan Partners
Board Member	✓ Osmol Therapeutics, Gilgamesh Pharmaceuticals
Advisor	✓ MindImmune Therapeutics, Manifest Sciences, Gate Neurosciences, INmune Bio

Burden of Depression

300M+

Antidepressant prescriptions filled annually

Major limitation of slow onset and incomplete response

30M+

Americans currently prescribed antidepressants

12.7%

US Population over 12 years old took antidepressants in prior month

7%

12-month prevalence of Depression in US population

25%

Remain ill one year after starting treatment

Almost two-thirds are on antidepressants for >2 years

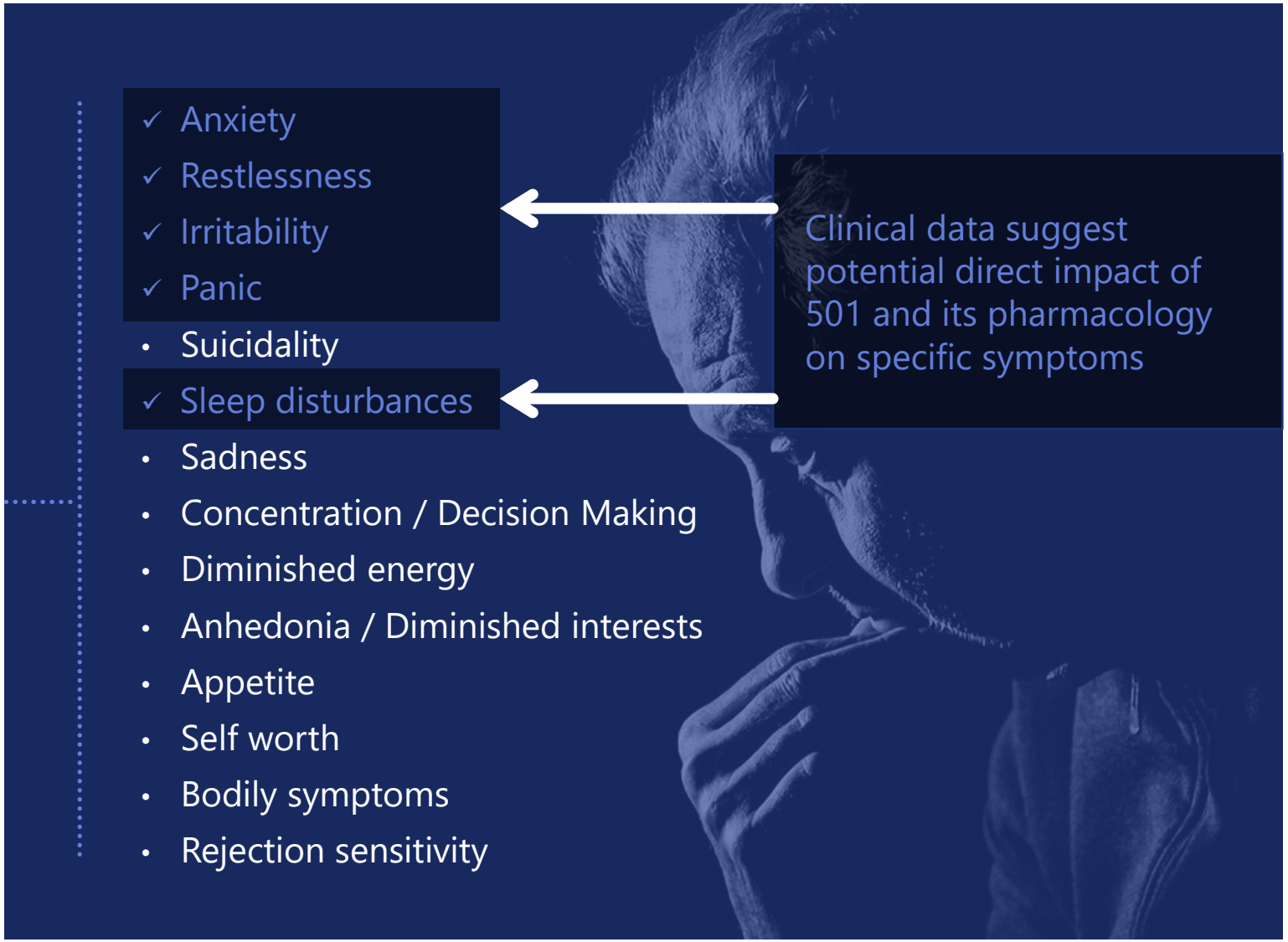
Sources: NIH/WHO, SAMHSA, NIMH
Pratt et al, 2017

What is depression?

- Operationalized diagnostic criteria defined by DSM-5
- Characterized by broad symptom expression

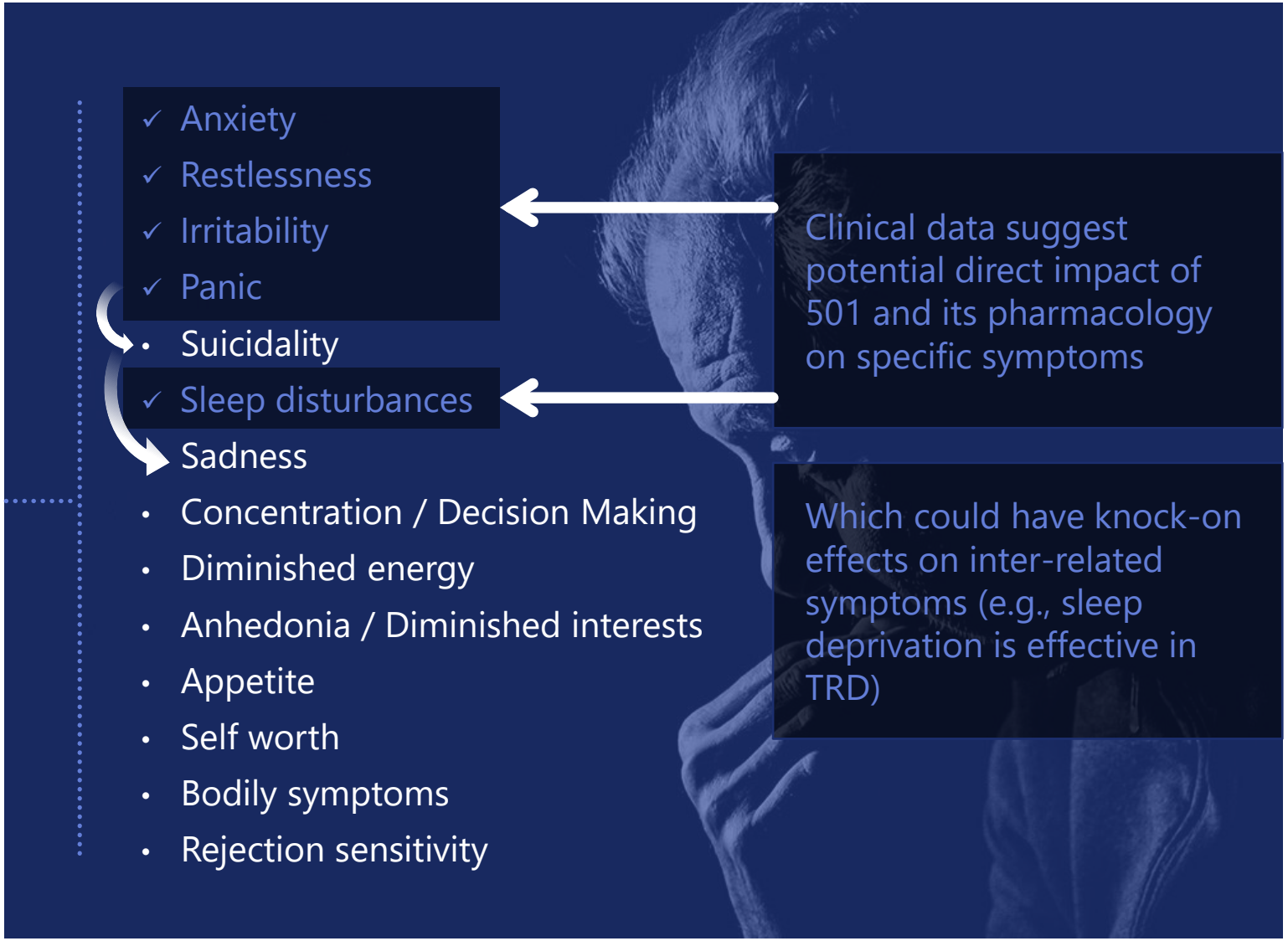
What is depression?

- Operationalized diagnostic criteria defined by DSM-5
- Characterized by broad symptom expression



What is depression?

- Operationalized diagnostic criteria defined by DSM-5
- Characterized by broad symptom expression



How to characterize Anxiety in Depression: Categorical vs Dimensional Definitions

50 – 85% of MDD

patients with moderate or great anxiety scale scores

40 – 70% of MDD

patients have had co-morbid anxiety disorders

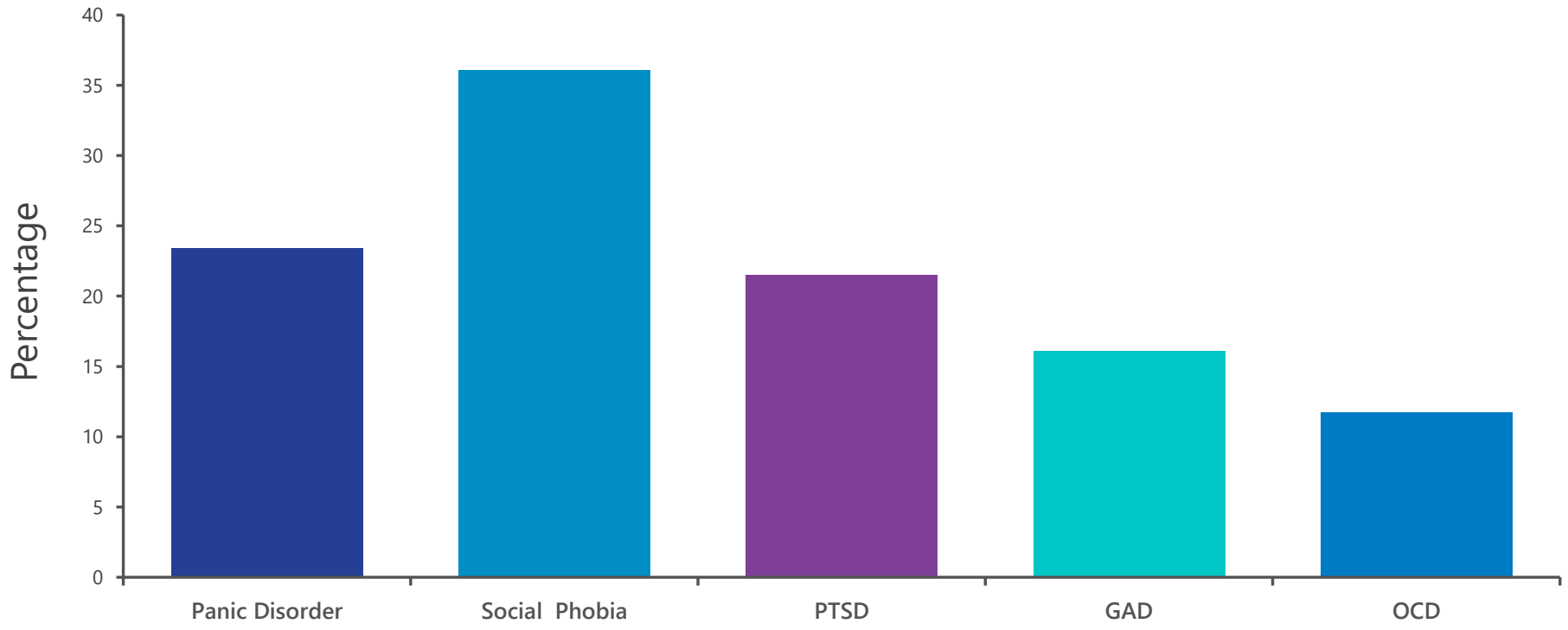
54 – 78% of MDD

Patients meet criteria for DSM-5 Anxious Distress

No matter how it is classified, the rates of anxiety are very high

Reviewed in Gaspersz et al 2017

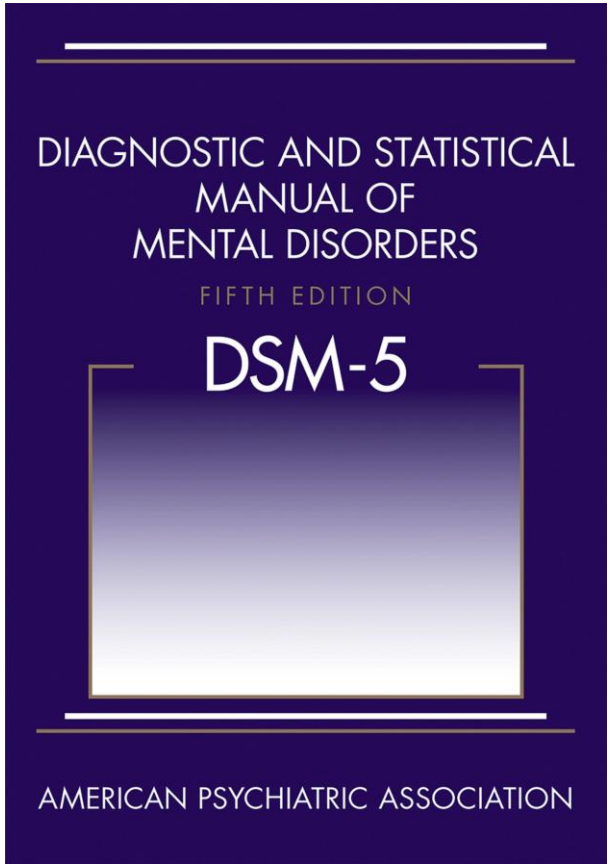
Comorbidities Among Individuals Diagnosed with Major Depressive Disorder



Zimmerman M, et al. *J Clin Psychiatry* 2002;63:187-193.

Major Depressive Disorder with Anxious Distress

- **According to DSM-5, for patients to meet the criteria of the anxious distress specifier, they must have at least 2 of the following 5 symptoms across a major depressive episode:**
  - feeling keyed up or tense
  - feeling unusually restless
  - difficulty concentrating because of worry
  - fear that something awful might happen
  - feeling that one might lose control of himself/herself
- **Compared to other classifications, Anxious Distress better identifies patients with poorer outcomes**



Clinical and Demographic Characteristics of Anxious Depression

- Greater severity of illness[1]
- Younger mean age[2]
- Earlier age of onset[2]
  - 20.6 ± 10.4 years in MDD with comorbid anxiety disorders
  - 28.4 ± 13.0 years in MDD alone
- Chronicity is common[3]
- Greater functional impairment[2]
- Increased risk of suicide[4]
- Greater chance of treatment discontinuation[5]

1. Joffe RT, et al. *Am J Psychiatry* 1993;150:1257-1258.
2. Fava M, et al. *Compr Psychiatry* 2000;41:97-102.
3. Van Valkenburg C, et al. *J Clin Psychiatry* 1984;45:367-369.
4. Clayton P, et al. *Am J Psychiatry* 1991;148:1512-1517.
5. Flint AJ, Rifat SL. *Am J Geriatr Psychiatry* 1997;5:107-115

Anxiety associated with poorer outcomes

Exemplified in STAR*D (53% with high level of anxiety symptoms)

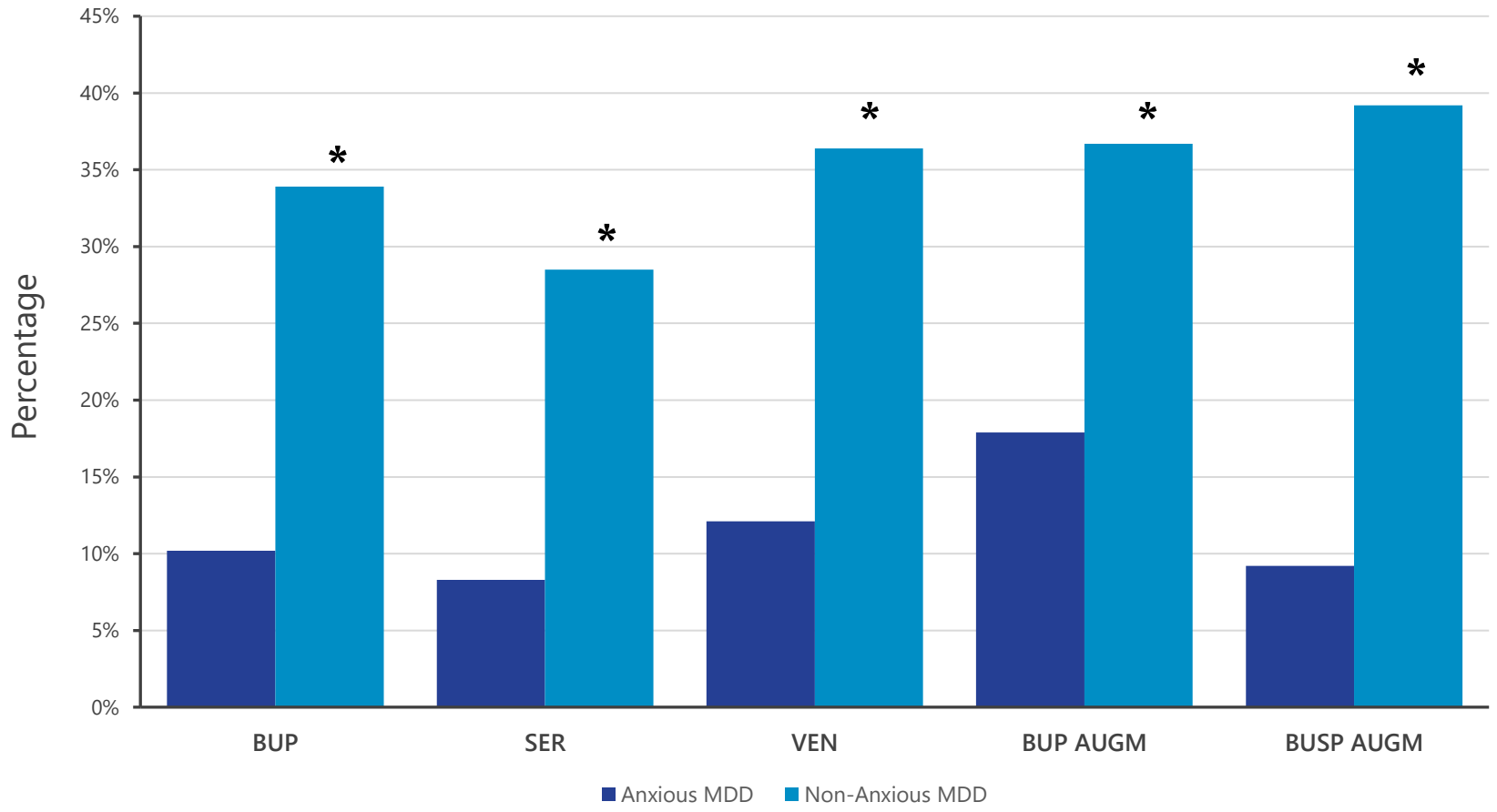
TABLE 2. Remission and Response in Patients in Level 1 of STAR*D, by Presence of Anxious Depression

Outcome	Anxious Depression				Total (N=2,876)		p	Adjusted p
	No (N=1,346)		Yes (N=1,530)					
	N	%	N	%	N	%		
Remission (score ≤7 on 17-item HAM-D)							<0.0001	0.0010[a]
No	896	66.6	1,190	77.8	2,086	72.5		
Yes	450	33.4	340	22.2	790	27.5		
Remission (score ≤5 on QIDS-SR)							<0.0001	0.0018[b]
No	822	61.1	1,105	72.5	1,927	67.1		
Yes	523	38.9	420	27.5	943	32.9		
Response (≥50% reduction from baseline on QIDS-SR)							<0.0001	<0.0001[b]
No	634	47.2	887	58.3	1,521	53.1		
Yes	709	52.8	634	41.7	1,343	46.9		
	Mean	SD	Mean	SD	Mean	SD		
QIDS-SR								
Exit score	7.9	5.4	10.2	6.1	9.1	5.9	<0.0001	<0.0001[b]
Change in score	–7.3	5.7	–6.8	6.1	–7.0	5.9	0.0298	<0.0001[b]
% Change in score	–46.6	35.3	–39.4	34.8	–42.8	35.2	<0.0001	<0.0001[b]

[a] Adjusted for regional center and baseline severity of depression (Hamilton Depression Rating Scale without anxiety factor).
[b] Adjusted for regional center and baseline severity of depression according to the Quick Inventory of Depressive Symptomatology–Self-Report.

Fava et al, Am J Psychiatry 2008; 165:342–351

Remission Rates (HAM-D-17 < 8) in Level 2 of STAR*D Anxious vs. Non-Anxious MDD



* $p < 0.05$

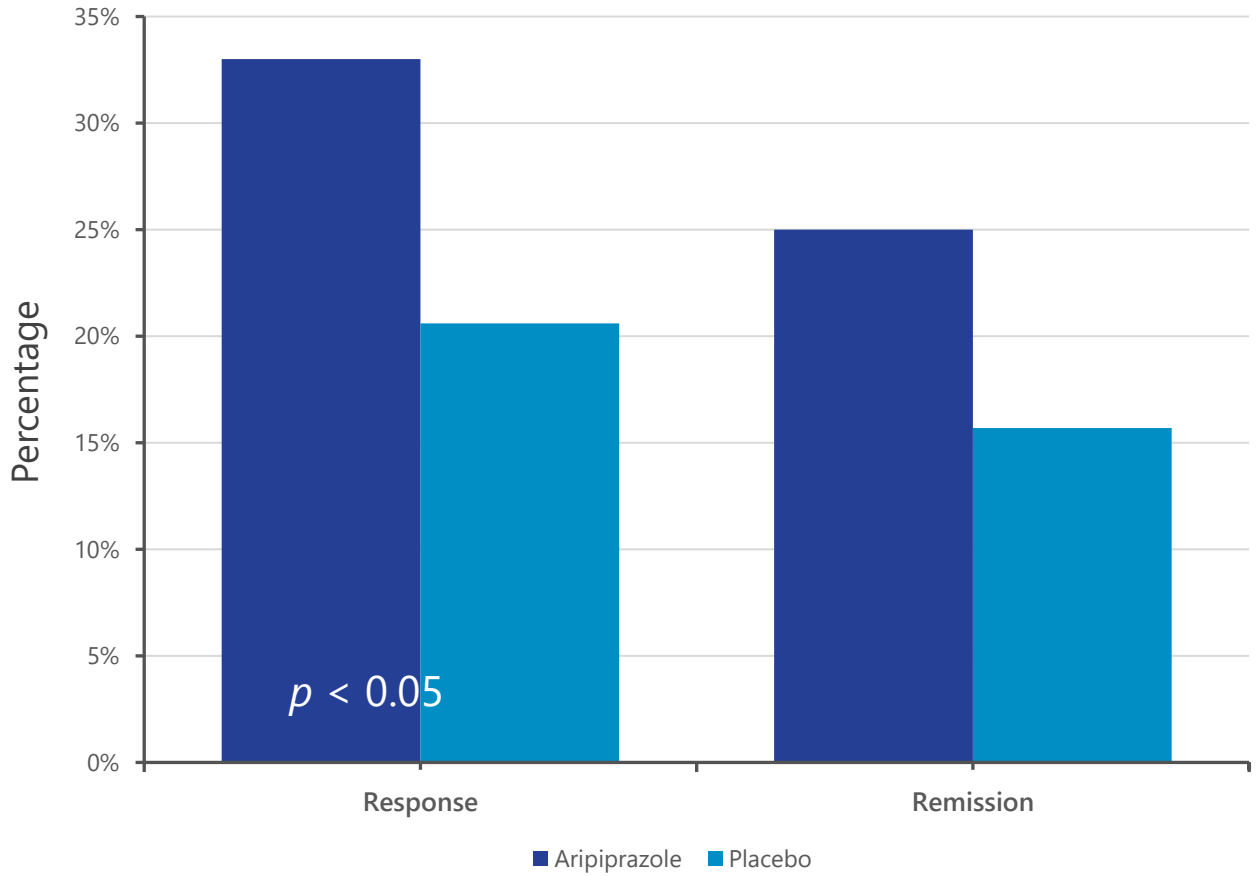
Fava M, et al. Am J Psychiatry 2008;165:342-351.

Treatment Approaches for Anxious Depression

- **No drug specifically approved for Depression with Anxious Distress**
- **Monotherapy with antidepressants**
  - Modestly effective
- **Augmentation often considered with**
  - Benzodiazepines
  - Atypical antipsychotic drugs
  - Buspirone, but no placebo-controlled evidence

Co-administration of Atypical Antipsychotics with Antidepressants

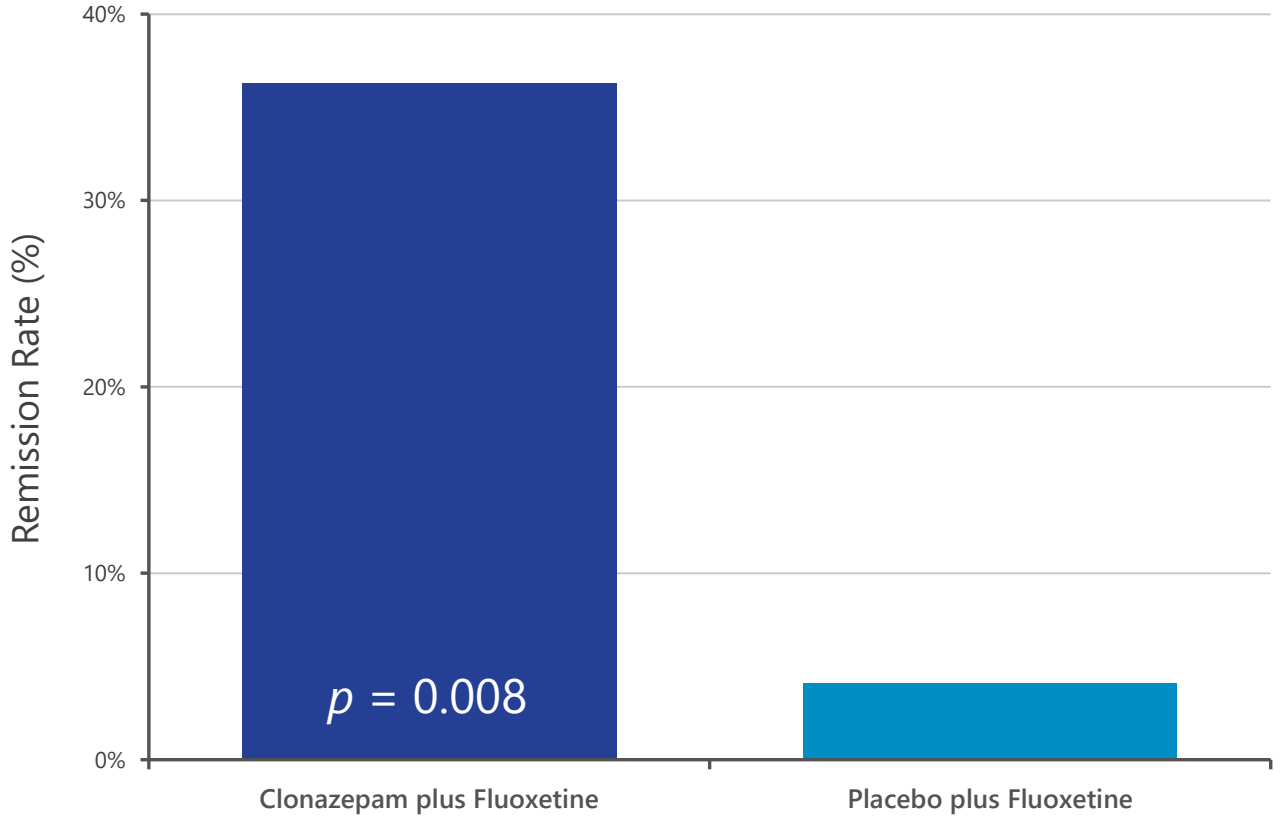
- **Concurrent use of atypical antipsychotics in anxious depression has been assessed with aripiprazole, quetiapine XR and brexpiprazole**
  - Efficacy demonstrated in anxious and non-anxious patients
- **Side effects of concern can include**
  - Akathisia
  - Weight gain
  - Somnolence/sedation
  - Metabolic disturbances



N = 435
* Trivedi MH, et al 2008

Co-administration of Benzodiazepines with Antidepressants

- **Concurrent use of benzodiazepines shown to confer rapid benefit (Furukawa 2001, Cochrane Review)**
  - Benefit noted from first through fourth week but not at week 6 and beyond
  - Trends for lower rate of discontinuation due to side effects
- **Caveats of benzodiazepine use**
  - Tolerance
  - Fall and accident risk
  - Cognitive dysfunction
  - Dependence / abuse potential



Papakostas et al 2010

Conclusions

- Anxious depression is very common (50 – 80%) and associated with significantly poorer clinical outcomes
- No treatments are specifically approved for this indication
- Clinicians typically address anxiety well after initiating antidepressant treatment with agents that are associated with a limiting side effect profile
- Clear, important need for proactively treating anxiety in depression
- BXC501 has solid rationale for development in the treatment of depression and its associated anxiety

Expansion of BXCL501 Development In Major Depressive Disorder

Robert Risinger, M.D.

Senior Vice President, Clinical Development

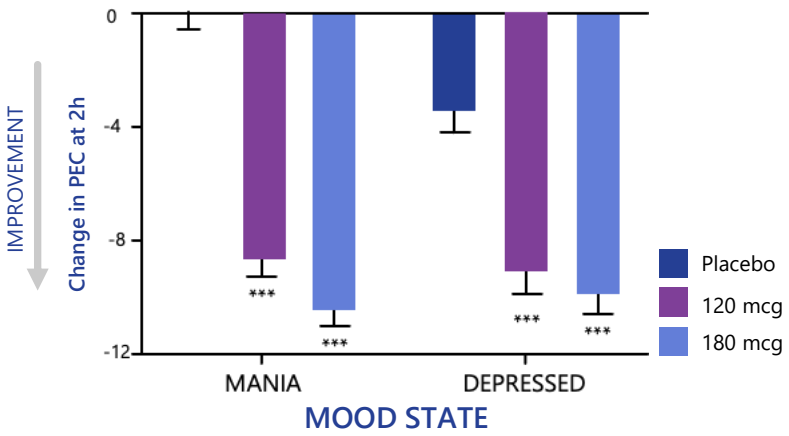
BXCL501 Program for Adjunctive Treatment of Patients with Depression

New Insights from Our Proprietary Clinical Data (SERENITY I and II, RELEASE)

- Revealed additional effects of addressing sympathetic hyperarousal with BXCL501
- BXCL501 demonstrated consistent reductions in agitation measures in depressed patients (bipolar; SERENITY II), schizophrenia and opiate withdrawal
- Clinical evidence supports further development of BXCL501 in MDD

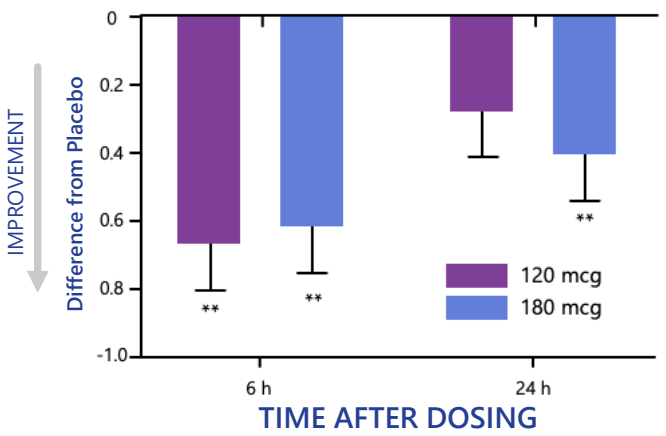
BXCL501 Market Expansion Opportunities by Leveraging Our Proprietary Clinical Data

BIPOLAR
(Serenity II)



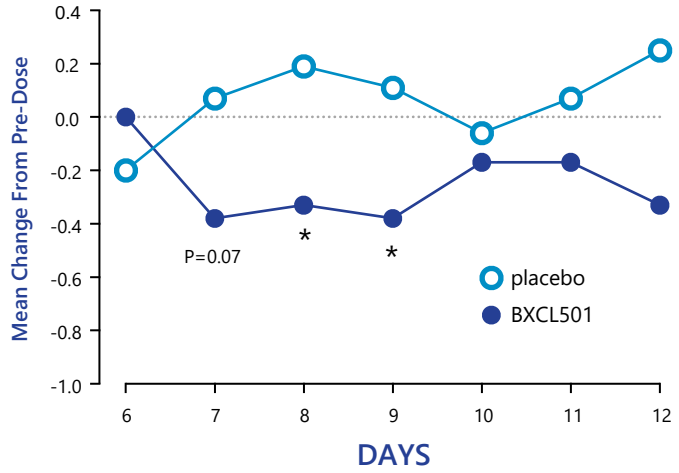
Significantly improved agitation in Depressed as well as in Manic

SCHIZOPHRENIA
(Serenity I)



Significantly improved Anxiety item of PANSS at 6 hours through next day for 180 mcg dose

OPIATE WITHDRAWAL
(Release)

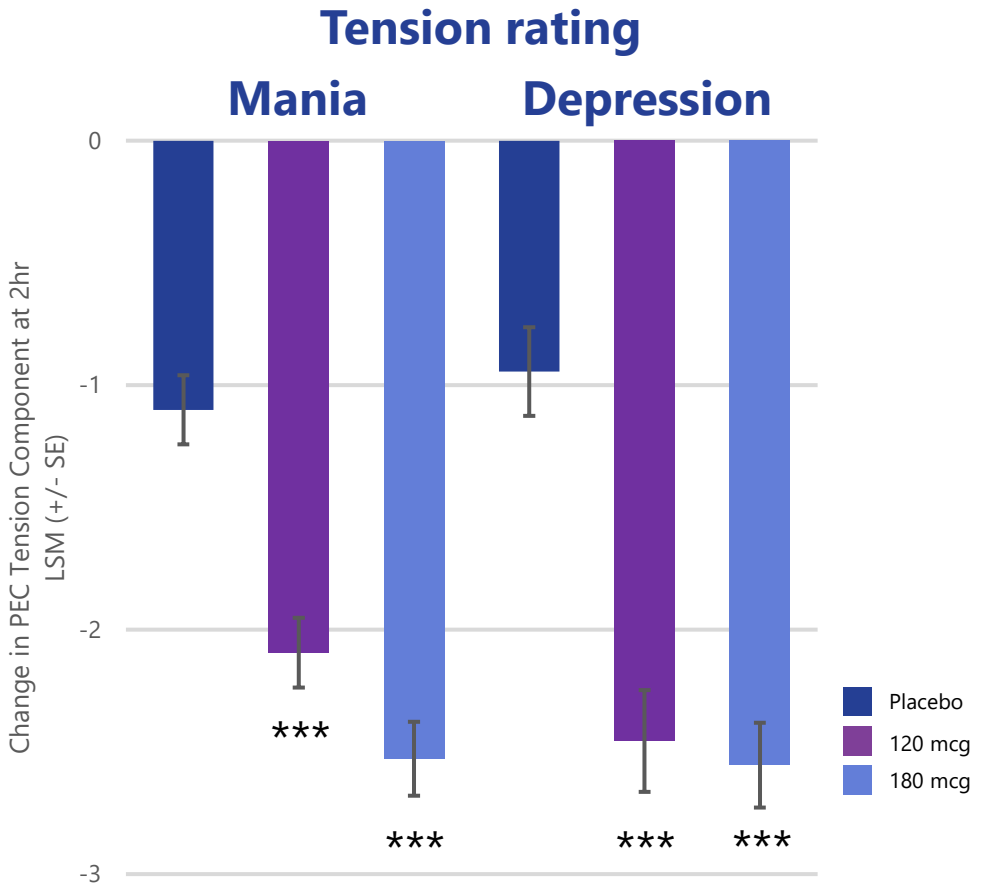


Improved clinician ratings of anxiety or irritability for 240 mcg twice daily for 7 days

BXCL501 may reduce anxiety-related symptoms in addition to agitation

Data Revealed Potential Value in Treating Depression

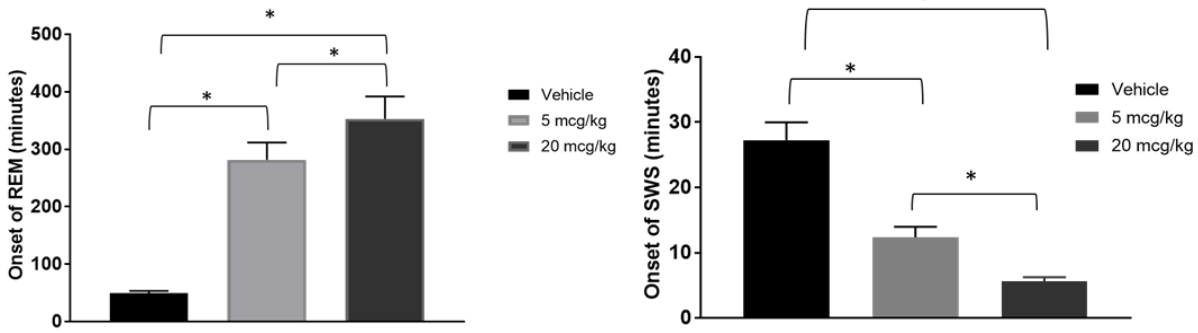
- SERENITY II (Bipolar Disorder) Study
- Besides agitation, BXCL501 has shown to be equally effective in reducing Tension in patients with depressed mood
- Suggesting development for rapid treatment of depression in patients with agitation and anxiety



PEC 'Tension' item rates apprehension, anxiety, nervousness, restlessness and panic
***p<0.0001

BXCL501: Calming and Restorative Effects Observed on Sleep Measures in Preclinical and Clinical Studies

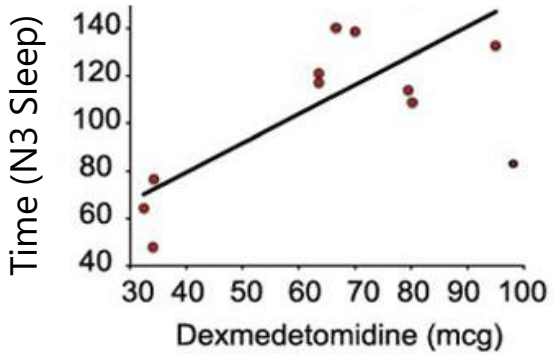
PRECLINICAL (RATS)



- Increased time to REM sleep
- Faster onset of slow wave sleep

(SWS is N3 in humans)

CLINICAL (HEALTHY VOLUNTEERS)



*Akeju et al.

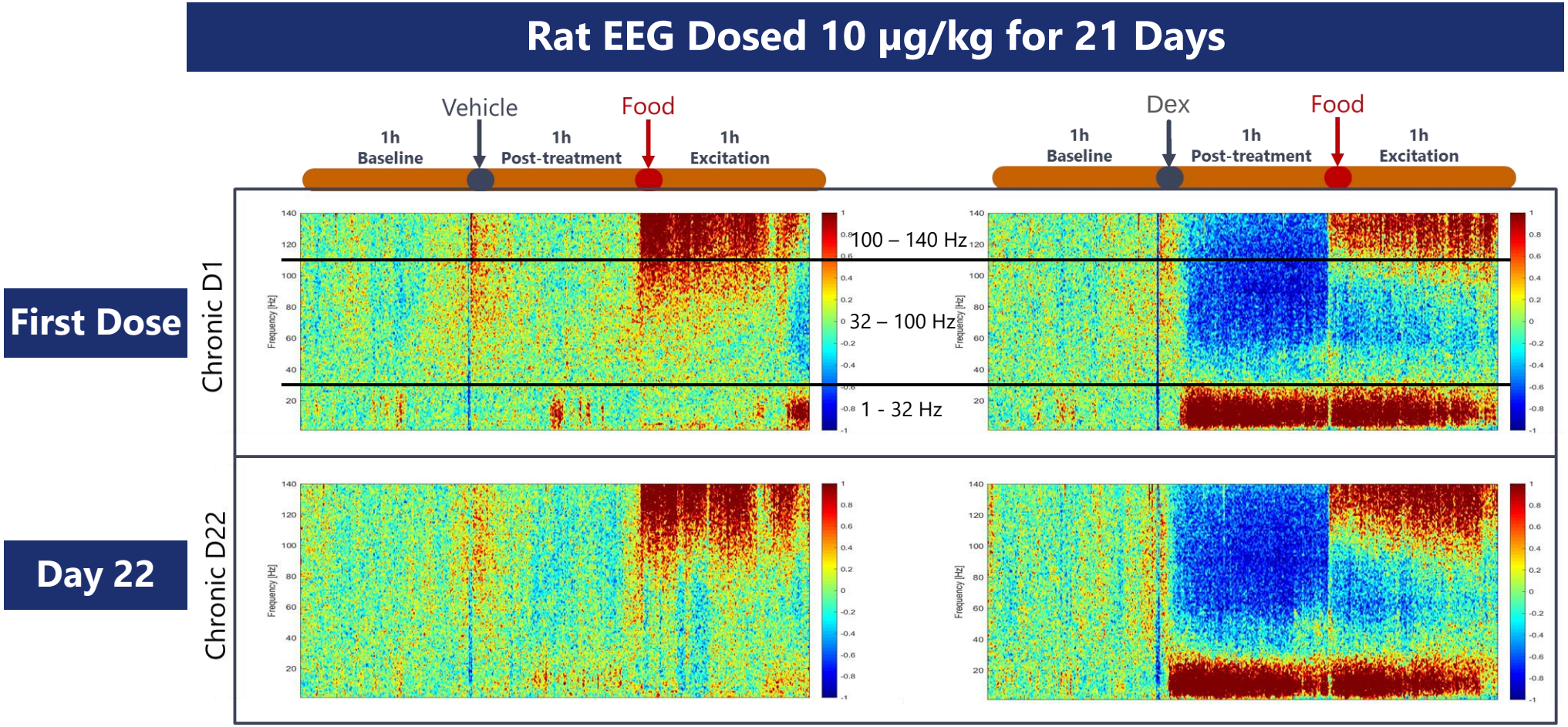
- Dose-related duration of slow wave sleep

In addition to reducing anxiety, BXCL501 also may **promote restorative sleep** in patients suffering from episodes of depression

Anxiety and sleep problems are not well treated by currently prescribed antidepressants

*Akeju et al., Clin Neurophysiol. 2018 Jan;129(1):69-78.

No Tolerance with Chronic Dosing of DEX (BXCL501)



Same EEG spectrum after 21 days indicates no tolerance or reduction in effect

Hamilton Depression Scale Items Potentially Addressed by BXCL501

BXCL501

1. DEPRESSED MOOD (sadness, hopeless, helpless, worthless
2. FEELINGS OF GUILT
3. SUICIDE (ideation)
4. **INSOMNIA: EARLY IN THE NIGHT**
5. **INSOMNIA: MIDDLE OF THE NIGHT**
6. **INSOMNIA: EARLY HOURS OF THE MORNING**
7. WORK AND ACTIVITIES
8. RETARDATION (slowness of thought and speech, impaired ability to concentrate, decreased motor activity)

BXCL501

9. **AGITATION**
10. **ANXIETY PSYCHIC**
11. **ANXIETY SOMATIC (physiological concomitants of anxiety)**
12. SOMATIC SYMPTOMS GASTRO-INTESTINAL
13. GENERAL SOMATIC SYMPTOMS
14. GENITAL SYMPTOMS (symptoms such as loss of libido, menstrual disturbances)
15. HYPOCHONDRIASIS
16. LOSS OF WEIGHT
17. INSIGHT

- Could address 6 HAMD-17 items
- May improve depressive symptoms (by impacting anxiety, agitation, sleep) poorly treated with SSRIs
- May rapidly alleviate core depressive symptoms over initial weeks of treatment, combined with SS/SNRIs

BXCL501 as a Potential Adjunctive Treatment in Depression

Planned MAD Study

HV

- Healthy volunteers, dosed daily
- Objectives: Assess safety, tolerability of daily doses of BXCL501

MDE Patients

- Depressed patients, dosed daily
- Objectives: Assess Safety and Tolerability in patients

Planned POC Trial in Depression

BXCL501 + SSRI

- Enroll patients with major depressive episode treated with SSRI or SNRI

Placebo + SSRI

- 4- to 6- week DB PC parallel group trial
- Objective: Antidepressant Efficacy & Safety of daily BXCL501

Meeting with FDA scheduled in Q4 2021 to discuss depression program

Q&A Period

Closing Remarks

Vimal Mehta, Ph.D.
Founder and CEO

Corporate Neuroscience Strategy

From First-in-Human Trial to NDA Filing for BXCL501 in Under 3 Years

Utilizing a robust AI platform to develop transformative medicines

Focusing on hard-to-treat neuropsychiatric symptoms by delineating underlying human biology and behavioral pathways

Leveraging re-innovation of clinical candidates and approved drugs

Delivering long-term stakeholder value through sustainable R&D pipeline

Thank You!